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Families' health care experiences for children with inherited metabolic diseases: protocol for a mixed methods cohort study

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ABSTRACT

Introduction: Children with inherited metabolic diseases often have complex and intensive health care needs and their families face challenges in receiving high-quality, family-centered health services. Improvement in care requires complex interventions involving multiple components and stakeholders, customized to specific care contexts. This study aims to comprehensively understand the health care experiences of children with inherited metabolic diseases and their families across Canada.

Methods and analysis: A two-stage explanatory sequential mixed methods design will be used. *Stage 1:* Quantitative data on health care networks and experiences with health care encounters will be collected from 100 parent/guardians through a care map, two baseline questionnaires, and 17 weekly diaries over 5–7 months. Care networks will be analyzed using social network analysis. Relationships between demographic or clinical variables and ratings of health care experiences across a range of family-centered care dimensions will be analyzed using generalized linear regression. Other quantitative data related to family experiences and health care experiences will be summarized descriptively. Ongoing analysis of quantitative data will inform sample selection for *Stage 2:* a subset of Stage 1 participants will participate in one-on-one videoconference interviews to elaborate on the quantitative data regarding care networks and health care experiences. Interview data will be analyzed thematically. Qualitative and quantitative data will be merged during analysis to arrive at an enhanced understanding of care experiences. Quantitative and qualitative data will be combined and presented narratively using a weaving approach (jointly on a theme-by-theme basis) and visually in a side-by-side joint display.

Ethics and dissemination: The study protocol and procedures were approved by the Children's Hospital of Eastern Ontario (CHEO)'s Research Ethics Board, the University of Ottawa

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Research Ethics Board, and the research ethics boards of each participating study center. Findings will be published in peer-reviewed journals and presented at scientific conferences.

Keywords: Family-centered care, pediatrics, healthcare experiences, inherited metabolic diseases, mixed methods

ARTICLE SUMMARY

Strengths and limitations of this study

- This study will ascertain family perspectives on health care networks and positive and negative care experiences for children with high care needs, such as those with inherited metabolic disease, forming a comprehensive understanding of current care, including gaps in family-centered care that will form the foundation for successful development of complex interventions to improve health care experiences for this understudied population.
- We expect this study to contribute to the methodological literature on assessment of health care experiences by using a novel combination of approaches, including care maps, diaries, and interviews.
- This study exemplifies partnership with patients and their families in co-designing research toward improved health care.
- A limitation of this study is the requirement of English proficiency for study participation, which will exclude a potentially more vulnerable population of children and families who, for example, require language supports for their health care.

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1 BACKGROUND

Inherited metabolic diseases (IMDs) are individually rare genetic conditions, often diagnosed in early childhood, that have a collective estimated global prevalence of 50.9 in 100,000 live births.[1] Many children with IMDs have complex and intensive health care needs.[2,3] Due in part to health service inequities related to infrastructure and funding, they and their families face multiple challenges in receiving high quality care[4] and, in common with children with medical complexities generally, may not receive optimal interdisciplinary family-centered services.[5,6]

Patient experience is a key pillar of a high performing health system.[7–9] Assessments of patient experience frequently address established principles of patient-centered care,[10] including access, coordination and continuity, and communication.[8,11,12] In pediatrics, these principles extend to family-centered care, emphasizing children's developmental needs and recognizing the central role of family members in disease management.[13,14] Families are often experts about the care needs of their children with rare diseases such as IMDs, underscoring the importance of their perspectives and their engagement in both health care and research.[5]

Several studies have focused on the quality of life and caregiving experiences of families of children with IMDs;[15–25] a smaller proportion have identified challenges or needs associated with providing and accessing care.[15,16,18–20] To begin to understand the health care experiences of this potentially underserved population, we completed two qualitative studies: first with representatives of relevant patient groups, then with caregivers of children with IMDs enrolled in a Canadian cohort study.[26,27] Overarching themes included a lack of familiarity with IMD care among many care providers outside of the metabolic clinic and poor suitability of some care systems to meet the needs of frequent and complex users. These studies expose a need for interventions that improve health care experiences of children with IMDs and their families. An Australian study found that families of children with IMDs experienced

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improved health care if care was accessed through a coordinating center.[28] Guidance about family-centered care for children with chronic conditions more generally suggests additional potential strategies for addressing some of these challenges, for example, co-developed care plans, receipt of care within a 'medical home', relational continuity with a key provider, improved collaboration between providers, and increased family involvement.[5,13,14,29,30] These potential strategies reflect *complex interventions*: each single strategy would require multiple interacting components, targeting multiple individuals or systems, and customization to specific contexts of care, with potential impacts on a range of outcomes.[31] Guided by the UK Medical Research Council (UKMRC) Complex Interventions Framework,[32,33] we have planned a rigorous, four-phase research program (Supplementary material 1) to develop complex interventions to improve family experiences with care. This protocol outlines our plans for "Phase I", the first study in our research program, in which we seek to build on our previous qualitative studies to more fully understand and describe the 'problem':[26,27,33] the nature, frequency, heterogeneity, and impact of positive and negative health care experiences of children with IMDs and their families. Such a purpose requires both quantitative data that can be generalized to a larger population and qualitative data to understand the nuances of individual experiences and is thus well-suited to a mixed methods design.[34] Mixed methods designs have been used in several studies of patient or family experiences in pediatric health care.[35–42]

1.1 Objectives

This study's overall aim is to comprehensively understand the health care experiences of children with IMDs and their families across Canada.

Quantitative objectives

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- To identify and describe the providers and services included in children's care networks and how they are connected to both the family and to one another, from parents' perspectives
- To prospectively measure the frequency, heterogeneity, and satisfaction with health care encounters of children and their families
- To identify the family characteristics and circumstances that form the context in which families experience health care, and their association with health care encounter satisfaction

Qualitative objectives

- To explain and enhance our understanding of:
    a. parents' perceptions and assessments of their children's care networks
    b. how families experience positive and negative health care encounters

Mixed methods objectives

To merge the quantitative and qualitative findings to arrive at an enhanced understanding of:

- The nature of children's care networks and how they are experienced and assessed by parents
- The family-centered elements and processes related to parent perceptions of positive and negative health care encounters

Pursuit of these objectives will be foundational to understanding how to develop complex, family-centered care interventions. For example, identifying the constellation of providers and services and their roles and connections in children's care networks may enable us to identify key providers for health care coordination interventions (quant, qual). Knowing the most

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frequently-used services will help with the prioritization of intervention development and implementation (quant). Understanding which aspects of care contribute to negative and positive experiences will help inform the creation of responsive interventions (quant, qual). An understanding of family characteristics and situations will shape interventions that account for the challenges and realities faced by families managing their child's care at home (quant).

The COVID-19 pandemic has exacerbated existing challenges related to access to care, and is expected to continue to affect how health care is delivered in the future. Therefore, we will collect data to understand the current context of health care delivery across Canada during the pandemic. In particular, we will aim to understand family experiences with virtual care, since this delivery modality has become more common due to pandemic response measures and the increase in its use is likely to influence health care delivery in a post-pandemic environment.

2 METHODS

2.1 Study design

The UKMRC Complex Interventions Framework, a phased approach to the design, evaluation, and implementation of complex interventions, guided this study's design.[32,33] Following previous studies of health care experiences,[43–47] we will also use the Picker Principles of Patient-Centered Care to provide a framework to guide data collection and analysis regarding key aspects of family-centered care.[12]

We will conduct a mixed methods study, following a two-stage explanatory sequential design (Figure 1).[34] **Stage 1:** Quantitative data will be collected on parent perceptions of children's health care networks (the people involved in a child's health care and how they are connected) and on health care encounters (frequency, context, experiences with care). These data

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will be analyzed on an ongoing basis to inform the sample selection for **Stage 2**: two subsets of participants from Stage 1 will participate in qualitative data collection (interviews) about (i) the participant's perception of the child's care network; and/or (ii) the factors that contributed to a strongly positive or negative health care experience. At the individual level data collection will be sequential: the quantitative collection of data related to the child's care network and experiences will precede the qualitative collection of data related to the network or to a specific health care experience. Data from both stages will be integrated during analysis. We will use the STrengthening the Reporting of OBservational studies in Epidemiology (STROBE) guideline[48] to report the study (Supplementary material 2).

2.2 Patient and public involvement

The interventions informed by this study will be complex, involving diverse systems, providers, and families, and aim to be family-centered. This underscores a need to engage families and providers,[49,50] especially in the context of rare disease where families become experts in their children's care needs.[35] Parents of children with IMD and adults living with IMD are engaged in this study. Three family/patient partners (IJ, NP, MS) are study co-investigators, leading the family engagement strategy, advising, and providing expertise, and sharing in decision-making at all study stages, from conceptualization to dissemination. The study also engaged 11 patient/family advisors, recruited through IMD family advocacy and support organizations, to provide advice and feedback during study instrument development; six of them also pilot tested the data collection instruments.

2.3 Quantitative sample

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Participants will be parents or legal guardians ("parents") of children diagnosed with an IMD. Although children's self-report of experiences is important, we seek to understand the experiences of health care for younger children (≤12 years). Parents are the family members most actively involved in seeking and managing health care for their children and thus are likely the best informants to provide comprehensive information on health care for this age group. For each participating family, one parent will be identified as the "designated parent" to provide data regarding one child in their family with an IMD ("designated child").

Eligibility criteria are described in Table 1. Child age will be restricted to ≤12 years as adolescents with chronic conditions have different health care and clinical treatment needs.[51,52] With respect to eligibility of IMD diagnoses, >1000 IMDs have been identified.[53] IMDs typically follow one of three broad clinical course trajectories, with different implications for health care usage and experiences: (a) chronic and generally non-progressive; (b) acute episodes of severe illness with or without accompanying chronic multi-system sequelae; and (c) progressive multi-system disease. Children with any of 30 priority IMDs included in an existing Canadian pediatric cohort study that will serve as one potential recruitment source[54,55] are eligible for this study (Table 1). Few of the IMDs included in that cohort study, however, are characterized as following trajectory (c). Thus, children will also be eligible for this study if they have an IMD that meets clinical criteria associated with trajectory (c) (Table 1), to be evaluated by clinician investigators on a case-by-case basis.

Table 1. Eligibility criteria

Inclusion	Exclusion
• The designated parent and designated child are Canadian residents • The designated child is ≤12 years at pre-screening • The designated child is receiving health care from one of 11 participating pediatric metabolic clinics across Canada: Alberta's Children Hospital, British Columbia Children's Hospital, Children's Hospital of Eastern Ontario, Health Sciences Centre Winnipeg Children's Hospital, The Hospital for Sick Children, IWK Health Centre, Kingston General Hospital, London Health Sciences Centre, McMaster Children's Hospital, Montreal Children's Hospital, Stollery Children's Hospital • The designated child has an IMD that is *either*	Designated parents who cannot speak, write, and read English comfortably

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1. identified in the following list:
   - ß-Ketothiolase deficiency
   - Arginase deficiency
   - Argininosuccinic aciduria
   - Carbamoyl phosphate synthetase deficiency
   - Carnitine uptake defect
   - Citrin deficiency
   - Citrullinemia
   - Farber disease
   - Galactosemia
   - Glycogen storage disease type 1
   - Glutaric acidemia type I
   - Guanidinoacetate methyltransferase deficiency
   - HMG-CoA lyase Deficiency
   - Homocystinuria
   - Hyperornithinemia-Hyperammonemia-Homocitrullinuria syndrome
   - Isovaleric acidemia
   - Long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency
   - Maple syrup urine disease
   - Medium chain acyl-CoA dehydrogenase deficiency
   - Methylmalonic acidemias
   - Mucopolysaccharidosis type I
   - Multiple carboxylase/biotinidase deficiency
   - N-acetylglutamate synthetase deficiency
   - Ornithine transcarbamylase deficiency
   - Phenylalanine hydroxylase deficiency
   - Propionic acidemia
   - Pyridoxine-dependent epilepsy
   - Trifunctional protein deficiency
   - Tyrosinemia type I
   - Very long-chain acyl-CoA dehydrogenase deficiency
2. *or* meets the following clinical criteria:
   - involves at least three organ systems *and*
   - chronic complications of the disease get progressively worse over time, even with available treatment

In order to collect data on health care experiences from a diverse sample of families, we will use a purposive, maximum variation sampling approach[56–58] to identify and recruit participants. We will aim for maximum variation on six selection variables on which experiences with care are anticipated to vary: study center, travel time from home to study center, child's sex**,** child's age (years), IMD type, and IMD typical clinical course trajectory. Treatment protocols and health care service availability and practice vary by IMD, clinical course classification, study center, and/or distance to specialists.[27,59] Health care encounters tend to be more frequent in the first years following an IMD diagnosis (usually in infancy) and parents characterize this time as uncertain and stressful.[27] Sex differences can affect metabolism, resulting in different care experiences for girls and boys.[60,61] We will prioritize the selection of participants who expect

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the designated child to have ≥1 health care encounter per month during the study to collect sufficient data for analysis.

2.4 Quantitative procedures

Participants will be recruited from the existing cohort study and/or from the study centers across Canada. Eligible parents will be notified of the study by the study team (by telephone) or by their associated study center (by telephone or at a clinic visit). For those notified by telephone, up to three contact attempts will be made. Participants will be enrolled on a rolling basis and the sample continually assessed for diversity on study selection variables to identify characteristics desired for further recruitment. Based on our previous experiences conducting studies with this population, we estimate a 50% response rate. Recruitment commenced in November 2020 and will be concluded when 100 families are enrolled. Interested parents will receive via email a postcard with study information and a link to the online Eligibility and Pre-Screening Questionnaire (5-10 min).

Data collection procedures are outlined in Figure 1. All questionnaires will be web-based. Study data will be collected and managed using Research Electronic Data Capture (REDCap) hosted at the Children's Hospital of Eastern Ontario (CHEO).[62,63] The participant, if they desire, may consult other family members, including the designated child, to complete the data collection tools. Children will continue to access health care normally. Participants will be reminded up to two times to complete each questionnaire.

2.5 Quantitative data elements and instruments

Data collection instruments are described in Table 2. Care map instructions, sample survey questions and measurements, and interview guides are provided in Supplementary material 3.

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Instruments were developed with input from clinicians, methodological experts, and family/patient partners and advisors, and pilot tested.

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Table 2. Data collection instruments

Data collection period Data instrument	Data type	Instrument completion time[a] (minutes)	Instrument and data details
Baseline			
Care Map	Quantitative	40	Participant creation of a care map of their perceptions regarding their child's network of care providers, which providers are perceived to work together to coordinate their child's care, and which providers are considered 'key providers' (maximum ten)
Care Map Questionnaire	Quantitative	5	Participant perceptions about: • Coordination of their child's care • Familiarity with their child by identified key health care providers
Baseline Questionnaire	Quantitative	20 – 40	Demographics and potential predictors of health care encounter satisfaction ratings, e.g., child health status, child and family characteristics, family resources in IMD management, and effects of the COVID-19 pandemic on child health and health care since March 2020
Pre-Questionnaire for Weekly Logs	Quantitative	5 – 20	Data will be used to tailor the Health Care Diaries, to reduce repetition of questions where responses are anticipated to remain constant over the study period
Follow-up			
Health Care Diaries[b]	Quantitative, qualitative	5 – 60	Descriptive data on health care encounters including: the mode of interaction, the care setting if applicable, the health care providers involved, the date of the encounter, financial costs, time inputs, and any parent-perceived effects of the COVID-19 pandemic (e.g., on scheduling or delivery of care) Optional, open-ended questions for descriptions of participant perceptions of care in each Picker Principle domain, and for the overall encounter The Experience Questionnaire will be tailored to each encounter's mode of interaction (in-person or virtual/remote), care setting, and context (planned or urgent care; whether it is a 'frequent' care encounter, as identified on the Pre-Questionnaire for the Weekly Logs)
Interviews	Qualitative	30 – 60 30 – 45	a) Map interviews: Seek to understand and elaborate on the care map, including how the participant selected providers to include on the map, the roles and relationships with the family for the providers designated on the map as "key providers", the meaning of connections drawn between providers, and how the participant feels about the effectiveness of the care network, including what improvements they see as potentially important b) Encounter interviews: Seek to clarify, interpret and deepen our understanding of information collected in the Health Care Diaries, specifically: elements of a health care encounter that contributed to participants' high or low satisfaction with that encounter; the impact of these experiences, especially the challenges, on the child, parent, other family members; and the context of general health care for their child (i.e., comparison between this encounter and past similar encounters). Impact will be iteratively defined, depending on the information shared by participants, and may include psychosocial, health, and/or economic impacts.

[a] Estimated

[b] All elements are completed once except the Health Care Diaries, which are completed weekly x 17 weeks

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2.5.1 Care maps

In this study, a 'care map' is a pictorial representation of the networks of health care providers around a child with an IMD and their family, commonly used in research on children with complex or chronic health conditions.[5,64–66] Guided by a set of instructions,[67] care maps will be drawn by hand, photographed, and uploaded to the study data collection database by the participant, and a digital version rendered by the study team.

2.5.2 Baseline questionnaires

Participants will be invited to complete three questionnaires: the Care Map Questionnaire, the Baseline Questionnaire, and the Pre-Questionnaire for Weekly Logs (content overview, Table 2). The Baseline Questionnaire also includes a number of validated instruments. Child health status will be assessed using the Child Health Questionnaire (CHQ-PF50)[68] for children ≥ 5 years or the Infant and Toddler Quality of Life Questionnaire (ITQOL-SF47)[69] for children <5 years. Both are parent-reported measures and have good validity and reliability.[69–71] Parent-perceived quality of life related to caring for the designated child will be measured using the CarerQol instrument. The CarerQol has good psychometric properties[72–75] and has been used with parents of children with chronic conditions, including rare diseases.[76–79] We reformatted the measure for online use.

2.5.3 Health care diaries

The Health Care Diary ("Diary") is composed of two parts: a Health Care Log and Experience Questionnaire. Once per week, participants will record whether a child had any health care encounters in a given week on the Health Care Log. If yes, they will complete an Experience Questionnaire for each of those encounters. Diary methods have been used in health studies to

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capture real-time information to reduce the recall errors associated with retrospective surveys,[80,81] with electronic diaries yielding higher quality data than paper diaries.[82,83] The definition of a health care encounter is provided in Figure 2. Evaluations will be made for the overall experience as well as in eight domains consistent with the Picker Principles of Patient-Centered Care where applicable:[84] access to care, information sharing, care coordination, physical comfort, emotional support, family involvement, respect for the patient/family, and continuity. The Consumer Assessment of Healthcare Providers and Systems Child Hospital Survey,[85] Ontario Emergency Department Patient Experience of Care Survey,[86] Outpatient Survey (Christine Kouri, Manager for Patient Experience, CHEO, e-mail communication, October 2017) and the Cost Utilization Survey for Child Phenylketonuria[87] were used as resources for our diary instrument development; diary questions were either author-developed, informed by, or adapted from these resources.

We will collect prospective data on blood draws done at home by the family, following the same family-centered care domains. For many IMDs, blood draws are essential to the ongoing monitoring of a child's health status, and though sometimes conducted by the family, require an ongoing dialogue with health care providers to adjust a child's medication, diet, or other treatment.

2.6 Qualitative sample

The two qualitative samples will be nested in the quantitative sample. Qualitative participants queried about their children's care networks must have completed the Care Map Questionnaire, and those queried about their positive or negative encounters must have completed at least four diaries. For the interview focused on the health care encounter ("encounter interview"), we will select participants who have had a health care encounter with which they reported they were

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"extremely satisfied", "extremely dissatisfied", or "somewhat dissatisfied" overall or on at least one family-centered care domain. We will use purposive, maximum variation sampling and extreme case sampling to separately sample participants for each interview set,[56–58] aiming for sample variation across the selection variables used for the quantitative sample and across health care settings in the encounter interviews. For the encounter interviews, if the parent who accompanied the child to the encounter is not the designated parent, they will be invited but asked to provide informed consent before proceeding. Some participants in the quantitative sample may be invited to participate in both interviews.

2.7 Qualitative procedures and data elements

On a rolling basis, participants will be identified and invited by e-mail to participate in a one-on-one, semi-structured interview held by videoconference or by audioconference, according to participant preference. For the interview focused on care network ("map interviews"), participants may be sampled at any time after completing the Care Map Questionnaire. For the encounter interviews, participants will be sampled during and up to three weeks after completing week 17 of the Diaries. Interviews will be audio-recorded with participant consent and transcribed. Up to three attempts to contact participants will be made to invite interview participation. Both interview sets will be semi-structured and informed by an interview guide.

2.8 Sample size

While we did not conduct a formal power calculation for the quantitative part of this study, given our largely descriptive purpose, we deemed a sample size of 100 families sufficiently large to support planned analyses across a heterogeneous sample, while maintaining feasibility for recruitment and study administration.

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Because of the duration and intensity of study participation, we anticipate some dropout. Dropout rates may increase with longer study lengths.[88,89] To facilitate participant retention, we pilot tested the feasibility of study questionnaires. In addition, we will: 1) enroll a new participant to replace any participant withdrawn before completion of at least four Diaries; 2) actively monitor completion of study instruments and follow up with participants if necessary; 3) provide participants with financial compensation (up to $400 in gift cards) for their time and as a participation incentive;[90] 4) encourage the scheduling of time each week to complete the Diaries; 5) allow for instrument completion over multiple sittings; and, 6) allow for flexibility of instrument completion.

A participant will be considered lost to follow-up upon notification of withdrawal or non-completion of an instrument within pre-specified timeframes; they will have the option to continue in the study if they proactively express a desire to do so. Data collected up to time of withdrawal will be included in the study.

The qualitative sample sizes will not be determined in advance; they will be assessed continuously and finalized during data collection. Information power is a methodological model for determining a qualitative sample size, and has five contributing dimensions related to: narrow vs broad qualitative objectives; the homogeneity of the sample on important characteristics; use of a theoretical framework; quality of interview data; and planned analytic strategy (case vs cross-case analysis).[91] Based on this concept and previous qualitative studies with parents of children with chronic conditions,[26,92–94] we anticipate a sample size of approximately 15-30 participants for each interview set.

2.9 Analyses

2.9.1 Quantitative analyses

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We will describe continuous variables using means and standard deviations or medians and interquartile ranges, and categorical variables using counts and proportions (%). Baseline data will be analyzed to describe the characteristics of participating families, including child and parent demographic variables, quality of life, experiences with managing an IMD in the context of COVID-19, and experiences with managing an IMD in general, including time and cost impacts.

From the care maps, children's networks of care providers and their interactions will be analyzed using an adapted form of social network analysis,[95,96] conducted using UCINET software.[97] We will describe who is in the network (nodes), identify the most common providers perceived as key providers, and analyze connections among providers from parents' perspectives (social network analysis calculations of network size and density and the degree centrality of providers).

From the Diaries, we will calculate the frequency (count and rate) of encounters by participant/child, accounting for follow-up time contributed. We will calculate counts and proportions to describe characteristics of health care encounters (e.g. pre-planned vs unplanned, the types of health care providers interacted with, care setting or mode of interaction), overall satisfaction ratings, and satisfaction ratings by Picker Principles (access to care, communication, coordination of care, etc).

To explore the potential relationships between a range of explanatory variables and satisfaction with health care experiences, depending on data quantity and distribution, we propose to use generalized linear regression analysis. The unit of analysis will be the individual health care encounter with each child having potentially different numbers of encounters. Explanatory variables will include both time-fixed and time-varying factors, namely child, family, and setting/provider characteristics (e.g., child age, IMD clinical course trajectory, travel

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time from home to care setting, socioeconomic status), health care setting, and mode of interaction. The five-point ordinal score for the overall experience of the health care encounter will be analyzed using ordinal logistic regression. Correlation in repeated measures on the same child will be accommodated either by directly modeling the covariance matrix or through the addition of child-specific random effects. A similar approach will be used to analyze the experience ratings within the eight family-centered care domains.

This study will minimize missing data by regularly monitoring completion of instruments and diary entries and following up with participants as necessary. Participants will have access to ongoing support from the study team. We will report on the number of missing values for each variable of interest, the reasons for missing values (if known), characteristics of participants with missing vs non-missing values for key variables, and missing data counts for each analysis. Our analytic strategy for managing missing data will depend on the extent of missingness of data for particular analyses and may rely on complete case analysis or multiple imputation. Withdrawn participants will be considered lost to follow-up at the date of their last completed baseline instrument or Health Care Diary.

2.9.2 Qualitative analyses

Guided by principles of family-centered care but also incorporating an inductive approach, we will use thematic analysis[98] to guide the coding and analysis of qualitative data across participants, using the following recommended process: 1) Review the interview transcripts and familiarize themselves with the data; 2) Do an initial, systematic coding of the data; 3) Identify themes of codes; 4) Review the generated themes against both the initial codes and the original data; 5) Refine the themes; and 6) Select and review extracts to illustrate the themes. We will

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repeatedly cycle through steps, particularly steps 3-4, to ensure the themes remain reflective of the original data.[98]

2.9.3 Mixed methods integration and analysis

The two types of data will be integrated at several points in the study. The quantitative data will be used to inform the qualitative sample as well as the interview questions and topics. We will compare the qualitative sample to the quantitative sample on the quantitative sampling selection variables. The quantitative and qualitative results will be merged in analysis and integrated to better understand the elements and processes related to health care networks and to positive or adverse health care encounters than would be gained from either data type alone.[34] In the final report, the qualitative and quantitative results will be integrated narratively using a weaving approach[99] (reported together, grouped by theme or concept) and presented visually in a side-by-side joint display with interpretations of the combined results and inferences about the meaning of the integrated data.[100]

3 DISCUSSION

This study will collect important information about parent perceptions about their families' experiences with health care for children with IMDs, a population with complex needs. Few pediatric studies have attempted to collect similarly comprehensive data on health care experiences.[41] Previous studies of children with IMDs and their families have focused on the impact of the IMD on the child, caregiver, or family well-being;[16–23] and/or on family experiences managing health care.[15,17,24] To our knowledge, this is the first broad study of health care experiences in pediatric IMD. We have designed a study that draws on mixed methods that best suit the research objectives, enabling the collection of experiential information

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of both breadth and depth. Diaries are an innovative tool in health research with potential for collecting real-time quantitative and qualitative data simultaneously. Care maps provide useful insight on how participants conceive of the networks of care around their children.

The main findings of this study will inform future phases of our research program, culminating in the co-development of family-centered interventions to improve health care for children with IMDs and their families. Comprehensive, prospective information collected on individual health care experiences will help elucidate the elements of health care that contribute to caregivers' negative and positive experiences. This information will also enable an assessment of the degree to which health care experiences are family-centered, ultimately helping to inform the creation of responsive interventions, especially for highly-frequented services. Care map data will identify key providers and enable an understanding of how participants perceive providers to be connected to each other and to the family. This may help to identify key providers who may be able to lead a child's 'medical home', playing an active role in coordinating their health care. Knowledge about the time, financial costs, and other inputs required to care for a child with an IMD is necessary to ensure that interventions are responsive to the realities of families for whom the interventions are designed to support. Data captured on health care experiences during the COVID-19 pandemic will contribute important information on the benefits and drawbacks of significant changes to health care delivery, such as virtual health care, that can improve the way that this care is delivered in the future. Through our larger research program, the evidence generated in this study will have a direct, actionable impact on family-centered health care for pediatric IMDs.

This study has limitations. All study data will be sought from parents. Their perceptions of their child's health care, for example, whether or not two providers work together to coordinate their child's care, may differ from providers' perceptions. However, health care providers will be

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interviewed about their perceived barriers to and facilitators of effective health care for children with IMDs in the next phase of the research program. Requiring English proficiency for study participation will limit the generalizability of study findings and will exclude a potentially more vulnerable population of children and families who, for example, require access to translators and additional supports as part of their care.

This study may be affected by selection and information biases. We will prioritize the selection of participants who expect the designated child to have multiple health care encounters during the study; our quantitative sample will be over-representative of families who are frequent health care users. This characteristic of our anticipated sample will increase the number of prospective health care experiences reported; however, it may limit the generalizability of quantitative findings. Past positive or negative experiences with care may motivate parents to participate in a study that provides the opportunity to share those problems and experiences. Non-response bias has been associated with both high and low patient satisfaction.[101,102] Parents whose children are experiencing urgent or critical health care issues, whose children are newly diagnosed (often associated with younger age), or who experience significant financial and time costs may feel overwhelmed and be less likely to participate or remain in the study than parents whose children's health issues are relatively stable.[26,103] We will attempt to minimize the burden of study participation by employing web-based data collection and offering compensation for study participation. To ensure that lack of home Internet access is not a barrier to study participation, participants may be loaned a study tablet with a mobile data plan to participate in the study. We anticipate that this may affect 10 - 15 participants.[104]

Factors such as recall and negativity bias may affect the reporting of all health care encounters. Our collection of prospective data via diaries, however, aims to capture experiences during all health care encounters, positive and negative, with a high frequency of reporting to

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mitigate errors associated with recall time.[81,105] The perspectives of the interviewer and data analysts may affect the collection and analysis of qualitative data. Interviewers will be trained by investigators with expertise in qualitative interviewing. Interviews will be transcribed as soon as possible after interviews and reviewed.

4 ETHICS AND DISSEMINATION

The study protocol and procedures were approved by CHEO's Research Ethics Board (#1955), the University of Ottawa Research Ethics Board, and the Research Ethics Boards of each participating study center. Participants will provide informed consent. Study data will be analyzed and stored securely.

Study findings will be published in peer-reviewed, open access journals and presented at relevant conferences. Additionally, a summary of study results will be shared with interested participants (opt-in). Study results will also inform future phases of our research to develop interventions to improve family-centered health care for this population.

FIGURES

Figure 1. Study design overview: mixed methods explanatory sequential design

Figure 2. Health care encounter definitions/eligibility

SUPPLEMENTARY MATERIALS

Supplementary material 1. Research program overview. Figure illustrating the overall research program and contextualizing this study (Study 1) within it

Supplementary material 2. Completed STROBE checklist

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Supplementary material 3. Summary of study questionnaires and instruments. List of study questionnaires with sample questions and copyrighted instruments used, care map instructions, and interview guides

DECLARATIONS

Availability of data and materials

Not applicable

Competing interests

SD has been or is a member of advisory boards for, received indirect educational grants from, and/or received indirect speakers' fees from Sanofi-Genzyme, Takeda, and Horizon Therapeutics. IDG is a recipient of a CIHR Foundation Grant (FDN# 143237). MP has been an advisory board member with honoraria from Ultragenyx (Mar/Apr 2021) and Horizon Therapeutics (Oct 2020), and received a speakers' honorarium (Sep 2020) and small investigator grant ($6,000, 2019) from Horizon Therapeutics. SS received educational grants from Biomarin, Shire, Recordati and serves/served as PI in clinical trials and postmarketing registries sponsored by Actelion, Biomarin, Shire, Ultragenyx. KW is the CEO of CANImmunize Inc. The remaining authors declare that they have no competing interests.

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Author contributions

BKP, PC, JB, EC, SD, LJG, CRG, JMG, RH, AJ, IJ, SK, JJMacKenzie, NM, JJMitchell, SGN, NP, AS, MS, KNS, RS, SS, MTeitelbaum, YT, CVK, JSW, BJW and KW conceived the study.

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AJC, BKP, RI, ML, KT, IJ, MS, NP, ZA, PC, JB, EC, IDG, CRG, SG, JMG, RH, AJ, SK, NM, JJMitchell, SGN, AP, MP, CP, LAP, AS, MTaljaard, MTeitelbaum, RS, SS, YT, and BJW designed and planned the study. AJC and BKP drafted the manuscript. AJC, BKP, RI, ML, KT, IJ, NP, MS, ZA, PC, JB, AC, EC, SD, LJG, SG, IDG, CRG, JMG, RH, SJG, AJ, SK, JJMacKenzie, NM, JJMitchell, SGN, AP, MP, CP, LAP, AS, KS, RS, KNS, SS, MTaljaard, MTeitelbaum, YT, CVK, JSW, BJW and KW reviewed/revised the manuscript. All authors approved the final manuscript.

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Guiding framework: Picker principles of family-centered care

Informs domains of data collection and analysis

	Aims	Sample	Data collection	Outcomes, products + integration
Quantitative	1. Identify & describe providers / services in care networks & their connections 2. Measure frequency & heterogeneity of, & satisfaction with, health care encounters 3. Identify family characteristics & circumstances associated with health care encounter satisfaction	100 parents	Baseline • Care Map • Care Map Questionnaire • Baseline Questionnaire Diaries • Weekly x 17	• Predictors of satisfaction with health care encounters • Frequency & features of + / - health care encounters • Impact of health care experiences on families • Key health care providers • Network & provider connectedness
Qualitative	1. Explain & enhance understanding of parents' perceptions & assessments of care networks	Subset 1 15-30 parents	Individual, semi-structured video/phone interview	• Description of key providers • Descriptions of care network connections • Assessment of care networks
Qualitative	2. Explain & corroborate understanding of how families experience positive (+) & negative (-) health care encounters	Subset 2 15-30 parents	Individual, semi-structured video/phone interview	• Factors influencing + / - health care encounters • Impact of encounters on families • Potential facilitators for improvement
Integrated	Merge quantitative & qualitative findings to arrive at an enhanced understanding of: 1. The nature of care networks & how they are experienced & assessed by parents 2. The family-centered elements & processes related to parent perceptions of + / - care encounters			Description of the: 1. Nature, parental experience, and parental assessment of care networks 2. Family-centered elements & processes related to parent perceptions of + / - care encounters

Inform purposive sampling
Inform qualitative data collection

Merge
Compare
Expand

Patient and family partners engaged in all project stages from study design to dissemination

Patient and family advisors and advisors engaged in instrument development and pilot testing

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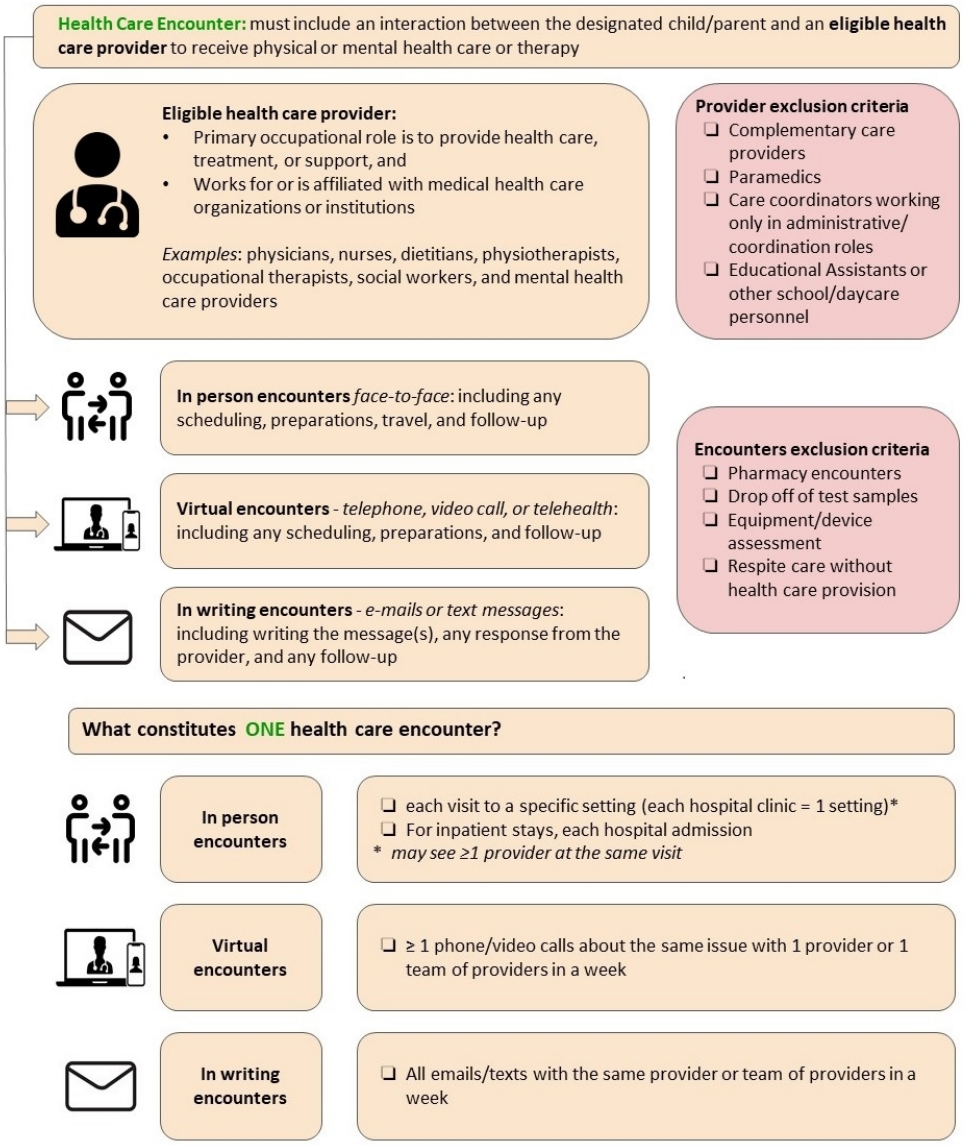

Figure 2. Health care encounter definitions/eligibility

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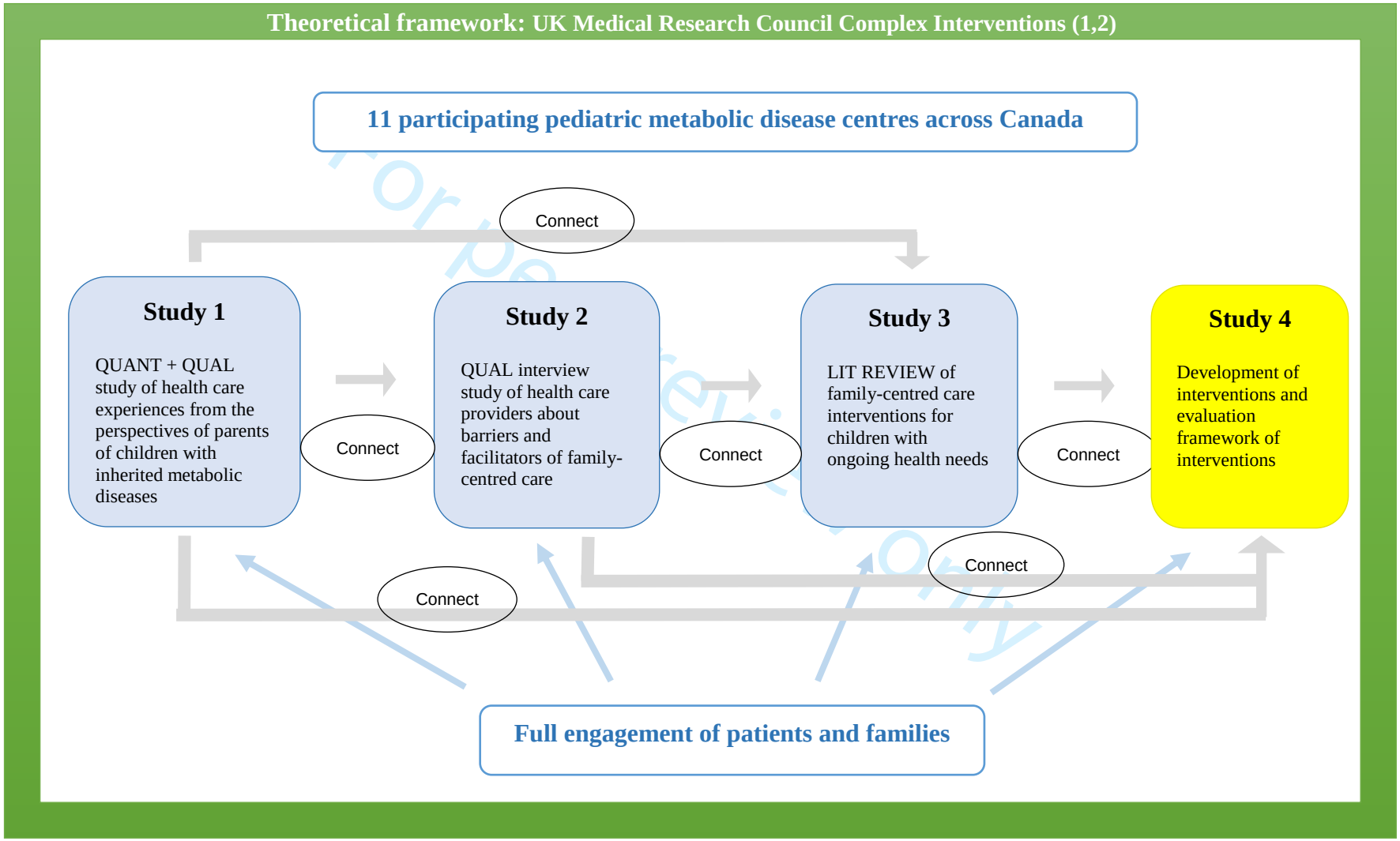
Supplementary material 1
Research Program Overview



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Supplementary material 2 – Completed STROBE checklist

	Item No	Recommendation	Page No
Title and abstract	1	(*a*) Indicate the study's design with a commonly used term in the title or the abstract	1
		(*b*) Provide in the abstract an informative and balanced summary of what was done and what was found	4
Introduction			
Background/rationale	2	Explain the scientific background and rationale for the investigation being reported	5 - 9
Objectives	3	State specific objectives, including any prespecified hypotheses	7 – 8
Methods			
Study design	4	Present key elements of study design early in the paper	9 – 10
Setting	5	Describe the setting, locations, and relevant dates, including periods of recruitment, exposure, follow-up, and data collection	13
Participants	6	(*a*) *Cohort study*—Give the eligibility criteria, and the sources and methods of selection of participants. Describe methods of follow-up *Case-control study*—Give the eligibility criteria, and the sources and methods of case ascertainment and control selection. Give the rationale for the choice of cases and controls *Cross-sectional study*—Give the eligibility criteria, and the sources and methods of selection of participants	10 – 13, 16 – 18, Figure 1
		(*b*) *Cohort study*—For matched studies, give matching criteria and number of exposed and unexposed *Case-control study*—For matched studies, give matching criteria and the number of controls per case	n/a
Variables	7	Clearly define all outcomes, exposures, predictors, potential confounders, and effect modifiers. Give diagnostic criteria, if applicable	13 – 16
Data sources/ measurement	8*	For each variable of interest, give sources of data and details of methods of assessment (measurement). Describe comparability of assessment methods if there is more than one group	13 – 17
Bias	9	Describe any efforts to address potential sources of bias	22 – 24
Study size	10	Explain how the study size was arrived at	17 – 18
Quantitative variables	11	Explain how quantitative variables were handled in the analyses. If applicable, describe which groupings were chosen and why	n/a
Statistical methods	12	(*a*) Describe all statistical methods, including those used to control for confounding	18 – 21
		(*b*) Describe any methods used to examine subgroups and interactions	19 – 20
		(*c*) Explain how missing data were addressed	20
		(*d*) *Cohort study*—If applicable, explain how loss to follow-up was addressed *Case-control study*—If applicable, explain how matching of cases and controls was addressed *Cross-sectional study*—If applicable, describe analytical methods taking account of sampling strategy	n/a
		(*e*) Describe any sensitivity analyses	n/a

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Results			
Participants	13*	(a) Report numbers of individuals at each stage of study—eg numbers potentially eligible, examined for eligibility, confirmed eligible, included in the study, completing follow-up, and analysed	n/a
		(b) Give reasons for non-participation at each stage	n/a
		(c) Consider use of a flow diagram	n/a
Descriptive data	14*	(a) Give characteristics of study participants (eg demographic, clinical, social) and information on exposures and potential confounders	n/a
		(b) Indicate number of participants with missing data for each variable of interest	n/a
		(c) *Cohort study*—Summarise follow-up time (eg, average and total amount)	n/a
Outcome data	15*	*Cohort study*—Report numbers of outcome events or summary measures over time	n/a
		Case-control study—Report numbers in each exposure category, or summary measures of exposure	n/a
		Cross-sectional study—Report numbers of outcome events or summary measures	n/a
Main results	16	(*a*) Give unadjusted estimates and, if applicable, confounder-adjusted estimates and their precision (eg, 95% confidence interval). Make clear which confounders were adjusted for and why they were included	n/a
		(*b*) Report category boundaries when continuous variables were categorized	n/a
		(*c*) If relevant, consider translating estimates of relative risk into absolute risk for a meaningful time period	n/a
Other analyses	17	Report other analyses done—eg analyses of subgroups and interactions, and sensitivity analyses	n/a
Discussion			
Key results	18	Summarise key results with reference to study objectives	n/a
Limitations	19	Discuss limitations of the study, taking into account sources of potential bias or imprecision. Discuss both direction and magnitude of any potential bias	22 – 24
Interpretation	20	Give a cautious overall interpretation of results considering objectives, limitations, multiplicity of analyses, results from similar studies, and other relevant evidence	n/a
Generalisability	21	Discuss the generalisability (external validity) of the study results	n/a
Other information			
Funding	22	Give the source of funding and the role of the funders for the present study and, if applicable, for the original study on which the present article is based	25

*Give information separately for cases and controls in case-control studies and, if applicable, for exposed and unexposed groups in cohort and cross-sectional studies.

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Supplementary material 3 - Summary of study questionnaires and instruments

A. Care map instructions

Making Your Child's Care Map

What is a care map?

A care map shows the people involved in your child's health care and how each person is connected to your child and to each other. An **example** is on **page 3.**

How to make your child's care map

The care map should reflect **how you see** your child's care, who's involved and how they're connected. There is no one way to create a care map. You can draw your own or use the template on page 4. It's up to you. Don't worry about getting it 100% right. If you would like, your child can help you draw the care map.

Things to remember

1. **Please do not put your child or other names on the care map.** Use "child," "family" and job titles instead.

2. When adding people or organizations that are part of your child's health care team. Group them together in a way that makes sense to you. See page 5 for examples of people and organizations that you could include. But there can be others!

3. **Try to include the people involved in your child's health care, not just organizations** (e.g. add teacher, Education Assistant, etc instead of just "school").

> What if my child sees 2 people with the same job title in the same clinic?
>
> 1) Label them Job Title #1, Job Title #2, etc.
>
> 2) Decide whether you consider one of them to be the **main "job title".** If yes, put a star next to Job Title #1. Example: if your child sees 2 metabolic physicians at the metabolic clinic, Dr. Chan, the one your child usually sees, and Dr. Singh, the one you see if Dr. Chan is away, label as follows: "Metabolic Physician #1★" and "Metabolic Physician #2". See Figure 1 below.

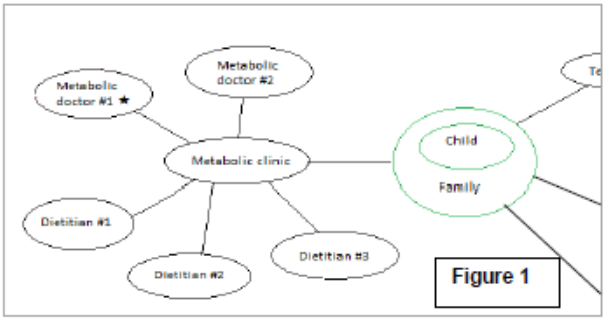

Figure 1

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4. **Connect providers:** Add lines to connect people or groups who work together for your child's health care, for example, by sharing information, providing or receiving referrals. People can be connected to others in same group or organization or at different groups. (See example, Page 3.)

> What if I don't know if 2 people work together or not?
>
> That's OK. Just draw the connections that you know about.

5. **IMPORTANT: Identify up to 10 key providers:** On the Care Map, put the letters "**KP**" next to that person's job title. **Key provider** = someone you think is key to your child's health care. If you do not think any of your child's caregivers is a key provider, just write "No key providers." (See example, Page 3.)

Once you are finished the care map

1. **Take a picture** of the care map or **save as a PDF** file. Make sure it is readable in the image.

2. To **upload the picture,** follow the steps in the email we sent you with this document. Please do not email the picture to the study team.

3. We will make a digital version of your care map. We will send you a link to view it and make sure that it is correct.

Questions?

If you have any questions while creating or uploading your care map, please **contact Andrea Chow**, study coordinator, at **(613) 562-5800 x4353**, or by email at **achow@uottawa.ca**.

Instructions adapted from: Antonelli, RC and Lind, C. Care Mapping: A How-To Guide for Patients and Families. http://www.childrenshospital.org/-/media/Care-Coordination/CareMappingforfamilies21813.ashx?la=en&hash=D8C02FCA893C9A29C939613532334E07127BF9E6. Accessed September 8, 2017.

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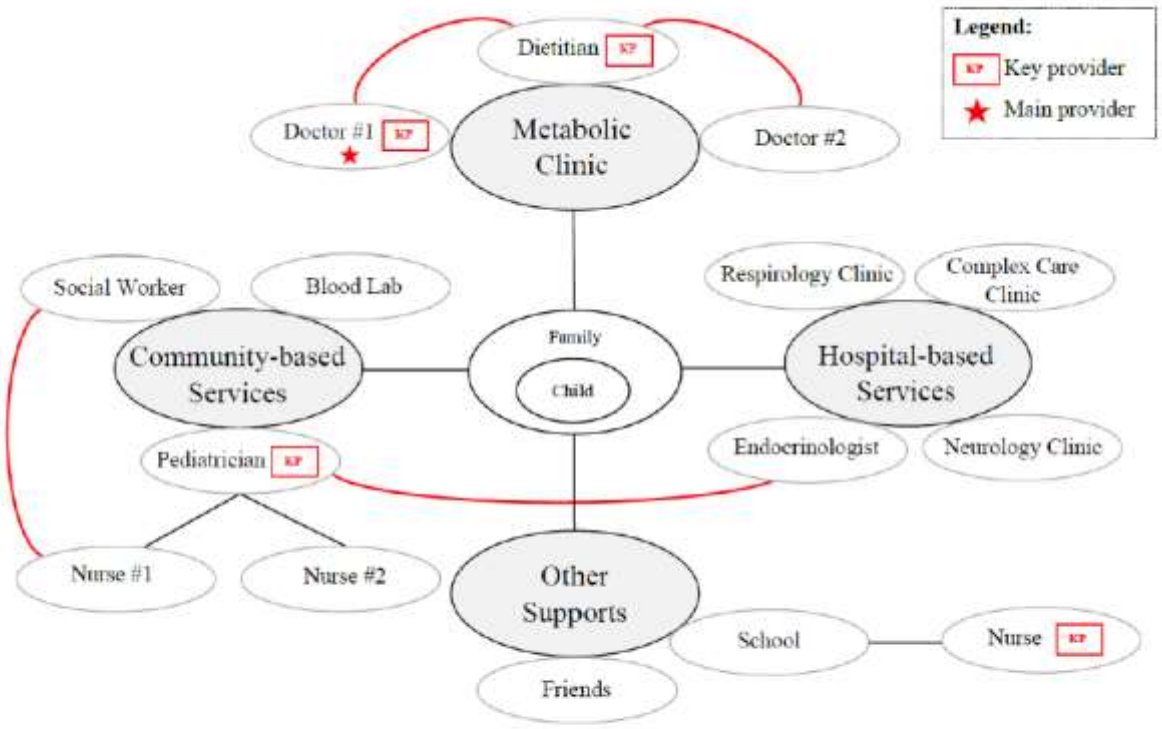
Care Map Example



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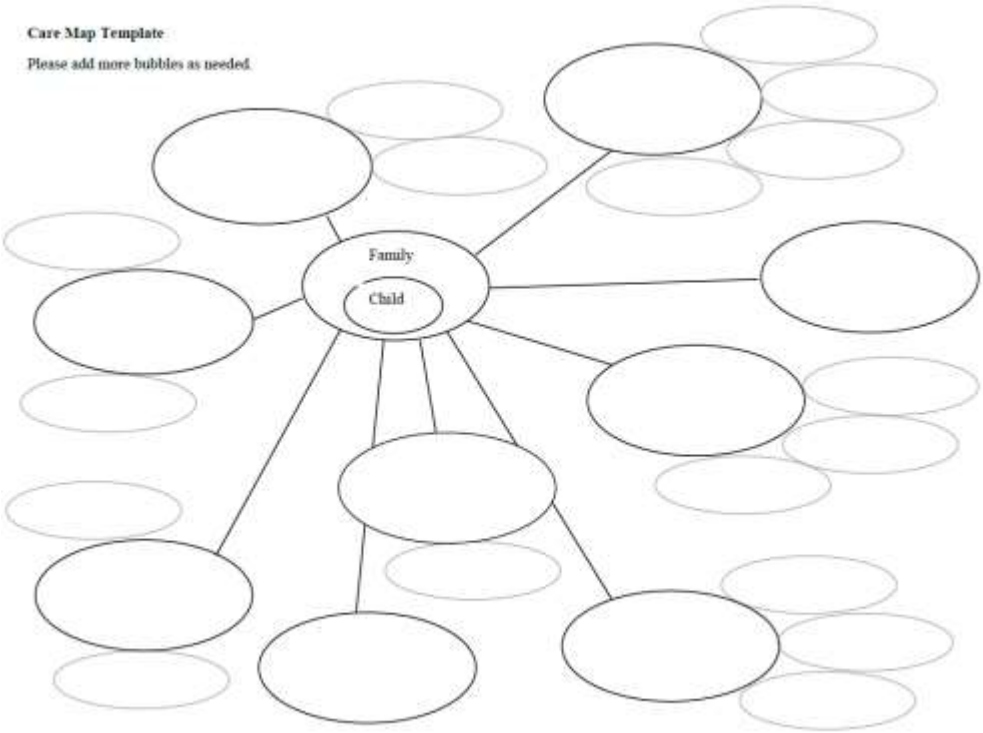
Care Map Template

Please add more bubbles as needed.



EXAMPLES

SETTINGS – IN HOSPITAL

Specialty Clinics
Audiology or Speech Therapy Clinic
Cardiology Clinic
Complex Care Clinic
Dental Clinic
Dermatology Clinic
Ear Nose Throat Clinic
Endocrinology Clinic
Gastroenterology Clinic
Hematology Clinic
Metabolic Clinic
Nephrology Clinic
Neurology Clinic
Optometry / Ophthalmology Clinic
Orthodontics Clinic
Orthopedic Clinic
Pediatrician's Clinic
Physiotherapy Clinic
Psychology / Psychiatry Clinic
Rehabilitation Clinic
Respirology Clinic
Rheumatology Clinic
Urology Clinic

Other
Ambulatory or Day Unit
Emergency Department
Feeding or Nutrition Clinic
Genetics Unit
ICU
Inpatient Unit
Laboratory
Mental Health / Counselling Services
NICU
Palliative Care Unit
Urologist
Radiology Unit

SETTINGS – IN HOSPITAL CONTINUED

Sleep Clinic
Social Work Unit

SETTINGS - IN COMMUNITY

Blood Lab
Clinic
Community Centre
Daycare
Diagnostic Imaging or other Laboratory
Hospice
Primary Health Care Clinic
School
Sleep Clinic
Walk-in or Urgent Care Clinic
Your Home

JOB TITLES

Acupuncturist
Audiologist
Behavioural therapist
Cardiologist
Care coordinator
Chiropractor
Complex care doctor
Counsellor
Critical care doctor
Dentist
Dermatologist
Dietitian
Doctor
Ear nose throat doctor
Educational Assistant
Endocrinologist
Family doctor

JOB TITLES CONTINUED

Gastroenterologist
Genetic counsellor
Geneticist
Hematologist
Homeopath
Lab technician
Massage therapist
Mental health professional
Metabolic doctor
Naturopathic doctor
Nephrologist
Neurologist
Nurse
Nurse practitioner
Occupational therapist
Ophthalmologist
Optometrist
Orthodontist
Orthopaedic doctor
Palliative care doctor
Paramedic
Personal support worker
Pediatrician
Pharmacist
Pharmacy assistant
Pharmacy technician
Physical therapist
Physiotherapist
Psychiatrist
Psychologist
Respirologist
Rheumatologist
Social worker
Speech therapist
Surgeon
Therapist

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B. Care map questionnaire

For each key provider identified on the Care Map, the following two questions are asked:

Question	Response options
How well does each of your child's key Health Care Providers know your child?	5-point Likert type scale
How well do you think your child's key health care providers coordinate your child's care with other providers?	5-point Likert type scale

C. Baseline questionnaire

Participants first complete either the **Child Health Questionnaire (CHQ-PF-50)** (if child age ≥ 5) or the **Infant Toddler Quality of Life Questionnaire (ITQOL-SF-47),** followed by author-developed questions, below:

Question	Response options
Your participating child	
What type of inherited metabolic disease does your child have?	Select one from list
What sex was your child assigned at birth?	Select one from list
In which province or territory does your family live?	Select one from list
At which metabolic clinic does your child currently receive the most care?	Select one from list
Does your participating child have any OTHER chronic illness or special needs?	Yes / No
Yes: To what extent do your participating child's special needs and/or chronic illnesses NEGATIVELY affect your family's emotional well-being?	Select one from list
Has your participating child had a major medical event or health crisis in the past two months?	Yes / No
Your child's caregivers	
INCLUDING YOU, how many primary caregivers does the participating child have?	Select # from list
For each caregiver:	
What is your relationship to your participating child?	Select one from list
What gender do you identify with most?	Select one from list
What is the highest level of education that you have COMPLETED?	Select one from list
What is your CURRENT employment status for paid work?	Select one from list
Are you or have you ever been a landed immigrant, permanent resident, or refugee to Canada?	Yes / No
Yes: In what YEAR did you first become a permanent resident, landed immigrant, or obtain refugee status in Canada?	Year
Participant completes the **Carer QOL-7D**	
Does your child have any secondary, UNPAID caregivers?	Yes / No
Yes: How many secondary UNPAID caregivers does your child have?	Select # from list
Does your child have any PAID caregivers?	Yes / No
Yes: How many paid caregivers does your child have?	Select # from list
Other members of your household	
Besides your participating child, how many children under the age of 18 live in your household ALL or SOME of the time?	Select # from list
1 or more: Besides your participating child, how many of the other 2 children in your household have the same inherited metabolic disease as your participating child?	Select # from list
Besides your participating child, do any of the OTHER [#] children in your household have any other chronic illness or special needs?	Yes / No
Yes: How much do the special needs and/or chronic illnesses of your OTHER child(ren) affect your family's resources (physical, financial, time, emotional, etc)?	Select one from list

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Besides your participating child, have any of your OTHER [#] child(ren) had a major medical event or health crisis in the past two months?	Yes / No
Do any of your [#] other children help to care for your participating child?	Yes / No
Yes: How many of the other # children in your household help to care for your participating child?	Select # from list
Managing the Inherited Metabolic Disease	
COVID-19 has changed the way that health care is provided. In general, how do you feel about the quality of your child's health care since the start of the pandemic (i.e., March 2020)?	5-point Likert type scale
Has your child been diagnosed with COVID-19?	Yes / No
Yes: When did they receive the diagnosis?	Month + year
Since then, have they needed extra health care because of their COVID-19 diagnosis?	Yes / No
Right now, do they still need extra health care because of their COVID-19 diagnosis?	Yes / No
How much do you agree/disagree with each statement for your child? Because of my child's COVID-19 diagnosis...	Matrix
...My child's well-being is worse	5-point Likert type scale
...My well-being is worse	5-point Likert type scale
...The well-being of other family member(s) besides me/my child is worse	5-point Likert type scale
Has anyone else in your family been diagnosed with COVID-19?	Yes / No
Over the past 6 months, how have the changes to health care and other services due to the pandemic affected your child's health care? Because of the pandemic...	Check all that apply: 1, One or more of my child's health care appointments or services were cancelled | 2, One or more of my child's health care appointments or services were delayed | 3, One or more of my child's health care appointments were changed from in-person to virtual (e.g., phone, video) | 4, I could not get to the lab, test centre, or pharmacy because their opening hours were reduced | 5, Only one primary caregiver was allowed to go with my child to a health care encounter | 0, None of the above
Checked any 1 – 5: Over the past 6 months, how have these changes to health care services due to the pandemic affected your child's CURRENT health or well-being? Because of the pandemic....	Check all that apply
Checked 1: What services or therapies were cancelled?	Check all that apply
Checked 2: What services or therapies were delayed?	Check all that apply
Checked 3: Overall, how did the virtual appointment(s) compare to similar in-person appointments before the pandemic (i.e., March 2020)?	5-point Likert type scale
Checked 3: Compared to similar in-person appointments before the pandemic (i.e., March 2020)...	Matrix
...the virtual appointment(s) were _____.	Shorter | the same | longer
...on the day of the virtual appointment(s), the wait for the provider was usually _____.	Shorter | the same | longer
...scheduling the virtual appointment(s) was _____.	Easier | the same | harder
...communicating with the provider during the virtual appointment(s) was _____.	Easier | the same | harder
...keeping my child comfortable during the virtual appointment(s) was _____.	Easier | the same | harder
...understanding what steps would take place after the virtual appointment(s) was _____.	Easier | the same | harder

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Checked 3: How was your privacy during the virtual appointment(s), compared to similar in-person appointments before the pandemic (i.e., March 2020)?	Select one from list
Checked 3: Did you feel more or less involved in decision-making about your child's health during the virtual appointment(s), compared to similar in-person appointments before the pandemic (i.e., March 2020)?	Select one from list
Checked 3: Compared to similar in-person encounters before the pandemic (i.e., March 2020), how much do you agree with the following statements?	Matrix
It was convenient to avoid travelling.	5-point Likert type scale
We were able talk to more than 1 provider at the same time.	5-point Likert type scale
The treatment was less effective.	5-point Likert type scale
It cost us less (out-of-pocket costs).	5-point Likert type scale
Checked 3: If the virtual appointment(s) were different in other ways compared to in-person appointments that took place before the pandemic (i.e., March 2020), please describe in the space below.	Open text
How much do you agree/disagree with each statement for your child? Because of the pandemic...	Matrix
...I avoided bringing my child to the emergency department or other parts of the hospital for treatment or care	5-point Likert type scale
...I avoided bringing my child to our primary care provider for treatment or care	5-point Likert type scale
...I had a hard time getting my child's medication or medical products	5-point Likert type scale
Because of the pandemic, I do not want my child to have in-person medical appointments	5-point Likert type scale
I worry about my child getting COVID-19	5-point Likert type scale
Compared to other children, my child is more at risk for COVID-19 complications because of their IMD	5-point Likert type scale
During the pandemic, I have taken public transportation or shared car services to take my child to in-person medical appointments. This has caused me stress or anxiety	5-point Likert type scale
During the pandemic, getting other health care-related needs for my child (e.g., supplies, medication) has caused me stress or anxiety	5-point Likert type scale
During the pandemic, managing my child's IMD at home has been more difficult	5-point Likert type scale
Since the start of the pandemic (i.e., March 2020), how has the pandemic affected your family?	Check all that apply
Do you have a plan, protocol or written directions from the metabolic clinic for managing your participating child's metabolic disease (e.g. a sick day protocol)?	Yes / no
OVER THE PAST 12 MONTHS, what types of treatments, therapies, services, products or equipment have you used to manage your child's inherited metabolic disease?	Check all that apply; specify further
For each item checked:	
OVER THE PAST 12 MONTHS, how hard was it to get [treatment, therapy, service, product, equipment]?	4-point Likert type scale
OVER THE PAST 12 MONTHS, did you get ENOUGH of [treatment, therapy, service, product, equipment]?	Got enough | Did not get enough
How difficult is it for you to manage this aspect of your child's care at home?	4-point Likert type scale
OVER THE PAST 12 MONTHS, were there services, therapies or products that your child needed to manage their IMD that you could not get WHEN they needed it?	Check all that apply
For each item checked:	
Why were the necessary medication or drugs not available when needed? Were the reasons:	Financial | Non-financial | Both
Financial or both: Please specify the FINANCIAL reasons why the [items] were not available.	Check all that apply
Non-financial or both: Please specify the NON-FINANCIAL reasons why the [items] were not available.	Check all that apply

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Over the past 3 months, how much time has your family (ALL TOGETHER) spent talking/writing to insurance companies or government agencies about health insurance coverage or reimbursement for medical products?	Select time band from list
If medical diet products used: Where do you TYPICALLY order your child's medical diet products (e.g. formula, supplements, medications, special foods) from?	Check all that apply
How do you typically RECEIVE your child's medical diet products?	Select one from list
How much time PER WEEK on average do you spend on getting medical diet products for your child (including ordering and pick up time)?	Select time band from list
Overall, how satisfied are you with the process of getting special diet products for your child?	5-point Likert type scale
Is there anything in particular that you like or dislike about your typical experiences with getting medical diet products? (Optional)	Open text
Does your family need to spend extra time planning and preparing special meals because of your child's inherited metabolic disease?	Yes / no
How much EXTRA time per WEEK on average does your family spend planning and preparing meals because of your child's inherited metabolic disease?	Select time band from list
If devices or therapies used, for each device or therapy:	
How much time PER WEEK on average does your family spend helping your child?	Select time band from list
Support services for family members	
OVER THE PAST 12 MONTHS, what type of support services for FAMILY MEMBERS have you used?	Check all that apply
For each service used:	
OVER THE PAST 12 MONTHS, how hard was it to get [service]?	4-point Likert type scale
OVER THE PAST 12 MONTHS, did you get ENOUGH of [service]?	Got enough | Did not get enough
Who helped your family to access [service] or told you about the service?	Check all that apply
OVER THE PAST 12 MONTHS, are there family support services that your family needed that you could not get WHEN you needed it?	Check all that apply
The impact of the inherited metabolic disease on caregivers' paid work outside the home	
OVER THE PAST 12 MONTHS, how many DAYS in TOTAL have your child's primary caregivers missed paid work due to caring for your participating child, for any reason?	Select one from list
Have any of your child's primary caregivers ever LEFT or QUIT a job because of your child's inherited metabolic disease?	Yes / no
Have any of your child's primary caregivers ever had to REDUCE their paid work hours because of your child's inherited metabolic disease?	Yes / no
The financial impact of the inherited metabolic disease on your family	
Over the past 12 months, what was your TOTAL household income before tax (Canadian dollars)?	Select one from list
OVER THE PAST 12 MONTHS, did you have to buy any products (including medical foods and formulas), devices, supplies, equipment or household items in order to manage your child's inherited metabolic disease?	Yes / no
If yes: How much, in Canadian dollars, did your family pay out of pocket to buy these products in the past 12 months?	Select one from list
OVER THE PAST 12 MONTHS, did you need to make any permanent or temporary modifications or renovations to your home to accommodate your child's inherited metabolic disease?	Yes / no
If yes: How much, in Canadian dollars, did your family pay out of pocket to modify or renovate your home in the past 12 months to accommodate your child's inherited metabolic disease?	Select one from list
If yes to either question re: purchase of products / home modifications: How difficult was it for your family to afford these recent costs (home modifications and/or products)?	Select one from list
Pharmacy encounters	

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In the past 6 months, how often did your family visit the pharmacy to pick up prescribed medications, foods, formulas or products for your participating child?	Select one from list
If more than once a week: In the past 6 months, how many different pharmacies did you visit?	Select one from list
If once or more:	
Where was the pharmacy (or pharmacies) located?	In Hospital / in community
ACCESS TO CARE Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
The medication or medical products typically arrived in the right formulation, supply amount, and in appropriate containers.	5-point Likert-type scale
The typical length of time between ordering the medication/medical products and picking them up was acceptable.	5-point Likert-type scale
The typical amount of time it takes to travel to the pharmacy was acceptable.	5-point Likert-type scale
Overall, how satisfied were you with your child's typical access to care at the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your child's typical access to care at the pharmacy? (Optional)	Open text
RESPECT FOR YOUR CHILD & FAMILY	
Overall, how satisfied were you with the respect that the pharmacist and/or staff typically showed you and your child over the past 6 months?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the respect that the pharmacy team typically showed you? (Optional)	Open text
COORDINATION OF CARE	
Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
Typically, the pharmacy team seemed to agree with each other about my child's treatment.	5-point Likert-type scale
Typically, the pharmacy team and providers at other locations coordinated my child's treatment appropriately.	5-point Likert-type scale
Did the metabolic clinic give you a letter about your child's inherited metabolic disease to share with the pharmacy?	Yes / no
Overall, how satisfied were you with the way that the pharmacy team typically coordinated your child's care?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the way that the care provider(s) typically coordinated your child's care? (Optional)	Open text
INFORMATION SHARING	
Overall, how satisfied were you with the typical information sharing by the pharmacy team over the past 6 months?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the typical information sharing by the pharmacy team? (Optional)	Open text
FAMILY INVOLVEMENT	
Overall, how satisfied were you with your family's typical involvement in your child's care at the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your family's typical involvement in your child's care at the pharmacy? (Optional)	Open text
FOLLOW UP AND CONTINUITY OF CARE	
Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
Typically, I got enough written information from the pharmacy about possible side effects of any new medications or any other new information I needed to take care of my child at home.	5-point Likert-type scale
Typically, I knew what to do or whom to call if I had any questions after leaving the pharmacy.	5-point Likert-type scale
Overall, how satisfied were you with the typical follow-up and continuity of care after visits to the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the typical continuity of care and follow-up after visits to the pharmacy? (Optional)	Open text

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OVERALL IMPRESSIONS OF THE PHARMACY	
Overall, how satisfied were you with your typical experiences with the pharmacy over the past 6 months?	5-point Likert-type scale
Is there anything else that you particularly liked or disliked about your typical experiences with the pharmacy? (Optional)	Open text

Indented questions are branched – only appear if specified responses to previous question(s) selected

D. Pre-questionnaire for the weekly diaries – sample questions

Question	**Response options**
Does your family do blood draws at home as part of managing your child's health?	Yes / no
If yes: How often do you and your child do blood draws at home?	Select one from list
Typically, what type of health care providers do you and your child interact with while getting the supplies, doing the blood draw, sending the sample, and waiting for and getting results?	Check all that apply
Where do you typically get the lancets you need for the blood draws?	Check all that apply
Considering your and your child's TYPICAL experience of doing blood draws at home, how much do you agree with the following statements:	
ACCESS TO CARE	
It is easy to get the items that we need to do the blood draws.	5-point Likert-type scale
If I have questions or concerns about doing a blood draw, I am able to contact the right care provider in a timely manner.	5-point Likert-type scale
The method we have to use to send the blood samples to the lab is acceptable (i.e. send by post, drop off in person).	5-point Likert-type scale
PHYSICAL COMFORT	
I receive enough support from the health provider(s) to make my child as physically comfortable as possible (i.e. to handle physical pain or discomfort) during the blood draw(s).	5-point Likert-type scale
EMOTIONAL SUPPORT	
If I share any concerns with the health care providers or staff, they respond appropriately.	5-point Likert-type scale
If my child shares any concerns with the health care providers or staff, they respond appropriately.	5-point Likert-type scale
We can do blood draws at a convenient time of the day for my family.	5-point Likert-type scale
I am comfortable drawing the blood from my child at home.	5-point Likert-type scale
INFORMATION SHARING	
I am able to share the information that I want to share about my child's blood draws with relevant provider(s).	5-point Likert-type scale
If I share information about my child's health, the care providers listen to what I have to say and respond appropriately.	5-point Likert-type scale
A care provider gives me information that I can understand about how to do the blood draw(s) at home, including getting supplies, doing the blood draw(s), and sending blood samples to the lab.	5-point Likert-type scale
A care provider gives me as much information as I want about the blood test results and clearly explains any recommendations for follow up.	5-point Likert-type scale
FOLLOW UP OF CARE	
The method that the clinic uses to send us the results of the blood tests is acceptable.	5-point Likert-type scale
The wait time for results from the blood tests is acceptable.	5-point Likert-type scale
Typically, how many days do you wait between sending the sample and receiving the results of the tests done on the blood draw?	Select one from list
YOUR FAMILY'S TIME INPUTS & FINANCIAL IMPACTS	
Typically, how much time do you and your child spend on EACH blood draw?	Select one from list

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Typically, do any of your child's caregivers have to take time off paid work to do a blood draw at home?	Yes / no
If yes: Typically, how much time off from paid work do your child's caregivers need to do a blood draw at home?	Select one from list
Does your family typically have any financial expenses that you have to pay directly because of, or related to, the blood draws you do at home, even if you are later reimbursed by an insurance plan?	Yes / no
If yes: What financial expenses does your family typically have?	Check all that apply
How much do you typically have to pay out of pocket and will NOT be reimbursed by a provincial or private insurance plan?	Select one from list
How much do you agree with this statement: The financial expenses related to doing blood draws at home typically cause me stress or anxiety.	5-point Likert-type scale

Indented questions are branched – only appear if specified responses to previous question(s) selected

E. Weekly diaries – sample questions

Question	**Response options**
Did your child receive any medical health care in Canada between [start_date] and [end_date]?	Yes / no
If yes: What types of health care encounter(s) did your child have during this week?	Check all that apply
FOLLOW-UP ON RECENT TESTS (if applicable)	
Did you expect a care provider to discuss the results of a medical test that your child had last week, in person, by phone or by e-mail?	Yes / no
If yes: With whom were you expecting to discuss the test results?	Select one from list
What type of test(s) were you waiting for the results of?	Check all that apply
How many days did you wait for a care provider to discuss the results for [test] with you?	Select one from list
How much do you agree with the following statement: The wait time for the [test] results was acceptable.	5-point Likert-type scale
How much do you agree with the following statement: A care provider gave me as much information as I wanted about the [test] results and clearly explained any recommendations for follow-up.	5-point Likert-type scale
If still waiting: How many days have you waited so far for a care provider to discuss the results for [test] with you?	Select one from list
How much do you agree with the following statement: The wait time so far for the [test] results is acceptable.	5-point Likert-type scale
How much do you agree with the following statement: A care provider gave me as much information as I wanted about where, when, and how I will get the [test] results.	5-point Likert-type scale
COVID-19	
Did your child get a COVID-19 test between [start_date] and [end_date]?	Yes/no
Was your child diagnosed with COVID-19 between [start_date] and [end_date]?	Yes/no
Were any health care encounters originally scheduled between [start_date] and [end_date] cancelled or delayed by the clinic or provider?	Yes/no

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Between [start_date] and [end_date], did you avoid seeking care for a health concern for your child due to the pandemic?	Yes/no
For every in-person encounter (questions and responses tailored to each care setting):	
Where did you and your child have this IN-PERSON care encounter?	At the Hospital / in community
Hospice or palliative care unit: Did your child stay overnight?	Yes/no
Was this encounter unplanned or pre-planned?	Select one from list
Were you and your child familiar with this place (e.g. clinic, lab, Hospital unit)?	Yes/no/somewhat
Was this place (e.g. clinic, lab, Hospital) in your province or territory of residence?	Select one from list
When did this encounter take place?	Date
During this care encounter, what type of health care provider(s) did you or your child see or communicate with?	Check all that apply
For each checked provider: Was this health care provider familiar with your child?	Yes/no/somewhat
Was this health care provider (or each of these health care providers or staff) familiar with your child's IMD?	Yes/no/somewhat
Lab: What type of tests did your child have during this encounter?	Check all that apply
If the participant identified this encounter's setting as a place where their child has frequent encounters in the Pre-Questionnaire for the weekly diaries:	
COMPARING THIS ENCOUNTER TO YOUR TYPICAL ENCOUNTERS	
Was this encounter the SAME as your typical encounters at [setting] in the following ways:	Check all that apply
The time you typically spend on encounters at [setting]: [participant response on Pre-Questionnaire]	
How long it took you to travel to the [setting]: [participant response on Pre-Questionnaire]	
Whether you or any of your child's other caregivers typically need to take time off paid work for encounters at [setting]: [participant response on Pre-Questionnaire]	
The time off paid work that you or any of your child's other caregivers typically need to take for encounters at [setting]: [participant response on Pre-Questionnaire]	
Whether your child typically misses school for encounters at [setting]: [participant response on Pre-Questionnaire]	
The time away from school that your child typically needs for encounters at [setting]: [participant response on Pre-Questionnaire]	
Your response to the statement "We are usually able to go to the [setting] at a convenient time in the day for our family": [participant response on Pre-Questionnaire]	
The time your child typically spend on encounters at [setting] (including arranging, the actual encounter, and any follow-up): [participant response on Pre-Questionnaire]	
For any aspect unchecked, the participant is asked about the aspect for this encounter.	

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Tests at the hospital laboratory (during overnight stays at the hospital, if applicable)	
During this hospital stay, did your child leave the [setting] to go to another area of the Hospital for medical testing? (e.g. radiology, imaging, diagnostics)	Yes/no
Yes: Did you or another caregiver go with your child when they had these tests?	Yes, always/yes, sometimes/no
If yes, always or sometimes: What type of tests did your child have outside the [setting]?	Check all that apply
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: We did not wait too long in the lab's waiting room.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: At the lab, information about the test process was shared with me in a way that I could understand.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: At the lab, age-appropriate information about my child's test process was shared with my child in a way that THEY could understand.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child had physical pain or discomfort during the test process, the lab's care provider(s) took the concern seriously and tried to address it.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child had physical pain or discomfort during the test process, the lab's care provider(s) respected my family's knowledge about how to make my child more comfortable.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child or I shared any concerns with the lab's health care providers or staff, they responded appropriately.	5-point Likert-type scale
Access to care	
How much do you agree with the following statements:	
We were able to schedule the encounter to take place at a convenient time in the day for my family.	5-point Likert-type scale
The length of time between getting a referral or scheduling the encounter and the date of the encounter was acceptable.	5-point Likert-type scale
The time it took to travel to the encounter was acceptable.	5-point Likert-type scale
We did not wait too long in the waiting room.	5-point Likert-type scale
The time spent waiting for the care provider was acceptable	5-point Likert-type scale
I was able to meet with the provider(s) I needed to talk to about my child's care.	5-point Likert-type scale
I (and/or my child) spent enough time with the health care provider(s).	5-point Likert-type scale
How long did it take you to travel from your home to this encounter?	Select one from list
Was this care encounter re-scheduled from a previous time that was cancelled or postponed?	Yes/no
Who cancelled or postponed the original encounter?	Select one from list

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Overall, how satisfied were you with your child's access to care for this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your child's access to care during this encounter? (Optional)	Open text
Coordination of care	
Did the metabolic clinic provide your family with an emergency department letter?	Yes/no
Yes: Did you share the letter with health care providers or staff at the Emergency Department?	Yes/no
How much do you agree with the following statements:	
During this health care encounter, an Emergency Department health care provider or staff read the letter and responded appropriately.	5-point Likert-type scale
During this health care encounter, the care providers seemed to work together.	5-point Likert-type scale
During this health care encounter, the care providers seemed to agree with each other about my child's care or treatment.	5-point Likert-type scale
Health care providers that we saw during this encounter and health care providers at other locations coordinated my child's care appropriately.	5-point Likert-type scale
Overall, how satisfied were you with the way that the care provider(s) coordinated your child's care during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the way that the care provider(s) coordinated your child's care during this encounter? (Optional)	Open text
Information sharing	
How much do you agree with the following statements: During this health care encounter (including during preparing for the encounter and any follow-up)...	
...information was shared with ME in a way that I could understand.	5-point Likert-type scale
...age-appropriate information about my child's treatment was shared with MY CHILD in a way that they could understand.	5-point Likert-type scale
...I was able to share the information that I wanted to share about my child's care with the provider(s).	5-point Likert-type scale
...if I shared information about my child's health, the care providers listened to what I had to say and responded appropriately.	5-point Likert-type scale
Overall, how satisfied were you with information sharing by health care providers and/or staff during this health care encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the information sharing by care providers and/or staff during this health care encounter? (Optional)	Open text
Physical comfort	
How much do you agree with the following statements: If my child had physical pain or discomfort during the health encounter...	5-point Likert-type scale
...the care provider(s) took the concern seriously and tried to address it.	5-point Likert-type scale

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...the care provider(s) respected my family's knowledge about how to make my child more comfortable.	5-point Likert-type scale
Overall, how satisfied were you with the care provider(s)'s efforts to make your child physically comfortable during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the care provider(s)'s efforts to make your child PHYSICALLY comfortable during this encounter? (Optional)	Open text
Emotional support	
How much do you agree with the following statements:	
If I shared any concerns with the health care providers or staff, they responded appropriately.	5-point Likert-type scale
If MY CHILD shared any concerns with the health care providers or staff, they responded appropriately.	5-point Likert-type scale
Overall, how satisfied were you with the health care providers' EMOTIONAL SUPPORT given to you and your child during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about efforts made by the health care providers or staff to provide EMOTIONAL support to you and your child during this encounter? (Optional)	5-point Likert-type scale
Family involvement	
Overall, how satisfied were you with your family's involvement in your child's care during this care encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the care provider(s)'s efforts to involve your family during this encounter? (Optional)	Open text
Respect for your child & family	
Overall, how satisfied were you with the respect that care providers and staff showed you and your child during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the respect that care providers and staff showed you and your child during this encounter? (Optional)	Open text
Follow up and continuity of care	
How much do you agree with the following statements:	
Before the end of this health care encounter, I got enough written information about possible side effects of any new medications, physical limitations, dietary needs or any other new information I needed to take care of my child at home.	5-point Likert-type scale
Before the end of the encounter, a care provider explained in a way that was easy to understand what symptoms or health problems to look out for after the encounter.	5-point Likert-type scale
I knew what to do or whom to call if I had any questions after this health care encounter.	5-point Likert-type scale
I got enough information about the next steps that I needed to take after the encounter. (e.g. booking new appointments, location of follow-up appointments, renewing prescriptions)	5-point Likert-type scale
The care provider(s) took all the steps that I expected them to take after the encounter. (e.g. making referrals, booking new appointments)	5-point Likert-type scale
Overall, how satisfied were you with the continuity of care and follow-up to this encounter?	5-point Likert-type scale

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Was there anything that you particularly liked or disliked about the continuity of care and follow-up to this encounter? (Optional)	Open text
Time inputs and financial impacts	
How much time did you and your child spend on this encounter (including arranging, travel if applicable, waiting, and the actual encounter)?	Select one from list
Did your family have any financial expenses that you had to pay directly because of, or in relation to, this care encounter, even if you were later reimbursed by an insurance plan?	Yes/no
Yes: What financial expenses did your family have?	Check all that apply
How much did you have to pay out of pocket and will NOT be reimbursed by a provincial or private insurance plan? Give your best estimate.	Select one from list
How much do you agree with this statement: The financial expenses related to this health care encounter cause me stress or anxiety.	5-point Likert-type scale
Did you or any of your child's other caregivers have to take time off paid work for this care encounter?	Yes/no
Yes: ALL TOGETHER, how much time off paid work did you need for this care encounter?	Select one from list
Did your child miss school/class for this care encounter?	Yes/no
Yes: How much time away from school/class did your child need for this care encounter?	Select one from list
Overall experience	
Overall, how satisfied were you with your and your child's experiences with care during this encounter?	5-point Likert-type scale
Was there anything else that you particularly liked or disliked about your and your child's experiences with care during this encounter? (Optional)	Open text
Compared to similar encounters that took place before the pandemic (i.e., March 2020), was this encounter shorter or longer?	Select one from list
Compared to similar encounters that took place before the pandemic (i.e., March 2020), was the amount of time from when you scheduled the appointment to the date of the appointment shorter or longer?	Select one from list
Did the provider request or tell you that there was a limit to the number of caregivers who could attend the encounter with your child?	Yes/no
Yes: Did this affect who or how many people went to the encounter with your child?	Yes/no
In your opinion, was there any other important difference between this encounter and other ones like it before the pandemic? If yes, please describe below.	Open text
Was this encounter scheduled BECAUSE it was required for a study or trial that your child is taking part in?	Yes/no
Which of your child's caregivers went to this encounter with your child?	Check all that apply
Who contributed to filling out this Experience Questionnaire?	Check all that apply
Which of these people was the MAIN person filling out this questionnaire?	Select one from list
Questions similar to these are also tailored to remote/virtual encounters, and for any blood draws done at home by the family.	

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F. Care Map Interview Guide

Overall Network of Care

1. Can you please walk me through your child's network of care?
    - *Probe for specific aspects related to:*
        - Validation of listed providers and connections – is the network accurate as it is or would you like to make any changes to it?
        - The process of drawing the network of care- how did you decide who to include in the network?

Identification of Key Providers

2. You identified [provider X] as a key provider. What are the factors that make them a 'key provider' for (kid's name)?
    - *Probe for specific aspects related to:*
        - From the care map questionnaire, I noticed that you indicated that this provider knows your child very well. What does that look like to you? (how do you know?)
        - How often does (kid's name) interact with the provider?
        - What is the provider's role in the child's care?

Care Coordination

3. You identified that [provider X] and [Provider Y] are connected. Can you tell me about that connection?
    - *Probe for specific aspects related to:*
        - What is the nature of the connection?
        - What is the impact of the connection on the family? How can you tell?
4. On the care map questionnaire, you told us that [provider X] coordinates with other providers "very well."
    - How does provider x work with other providers (e.g., shares information, makes referrals, you don't have to fill them in on Can you tell me about factors that influenced your positive rating?
5. On the care map questionnaire, you told us that [provider X] coordinates with other providers "not well at all."
    - Can you tell me about factors that influenced your negative rating?
    - What could/should be done to improve it?

Adequacy of Network of Care

6. How well does this network of care meet your child's needs? How does this network of care meet your needs?
    - *Probe for specific aspects related to*
        - Are there parts of the network that work better than others? What parts work better? In what ways?
        - What can be improved in this network of care? How could the network be improved to better meet (kid's) needs?
        - Are there people who should be key providers but they are not listed as such? Who and How come?
        - Are there providers who should be connected on your care map but who are not currently connected? Which providers do you think should be connected? How would this help?

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G. Encounter Interview Guide

Direct contributors to satisfaction rating

1. You rated your satisfaction with [this encounter / specific Picker Principle] [RATING]. In your view, what made this interaction [positive/negative]?
    - *Probe for specific aspects related to:*
        - Picker Principles
        - Setting
        - Modality

Identification of how negative encounter could have been different

2. *If negative:* In your opinion, what would have made this encounter better for you and your child?
    - *Probe for role of:*
        - Specific providers / teams
        - Specific actions (actor not necessarily important)

3. *For each agent of change:* In your opinion, what could they have done differently?

Identification of HCP who could have helped

4. *If negative:* Is there another health care provider involved in your child's care who you think could have helped in this situation?

Comparison to previous, similar encounters (same mode)

5. Have you been to [setting] before? / Have you met this [provider] before?
    - *If yes:* How did this interaction compare with other interactions you've had [with PROVIDER/at LOCATION]?
        - *If worse or better:*
            - How was it worse/better?
            - Was there anything else different about this encounter than other ones (e.g., longer wait time, different receptionist)?
        - *If the same – negative:* What do you wish would happen instead?

Impact of the encounter

6. How did this interaction affect your child, you, and other members of your family?
    - *Probe for different impacts*, e.g., psychological, physical, emotional, social, financial
    - *If negative:* What / is there anything else that would have made this interaction more positive for you?
    - *If negative and other encounters are the same:* Since you've had other negative experiences [at clinic / with provider], did it change the way you prepared for this encounter?

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BMJ Open

Families' health care experiences for children with inherited metabolic diseases: protocol for a mixed methods cohort study

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ABSTRACT

Introduction: Children with inherited metabolic diseases (IMD) often have complex and intensive health care needs and their families face challenges in receiving high-quality, family-centered health services. Improvement in care requires complex interventions involving multiple components and stakeholders, customized to specific care contexts. This study aims to comprehensively understand the health care experiences of children with IMD and their families across Canada.

Methods and analysis: A two-stage explanatory sequential mixed methods design will be used. *Stage 1:* Quantitative data on health care networks and encounter experiences will be collected from 100 parent/guardians through a care map, two baseline questionnaires, and 17 weekly diaries over 5–7 months. Care networks will be analyzed using social network analysis. Relationships between demographic or clinical variables and ratings of health care experiences across a range of family-centered care dimensions will be analyzed using generalized linear regression. Other quantitative data related to family experiences and health care experiences will be summarized descriptively. Ongoing analysis of quantitative data and purposive, maximum variation sampling will inform sample selection for *Stage 2:* a subset of Stage 1 participants will participate in one-on-one videoconference interviews to elaborate on the quantitative data regarding care networks and health care experiences. Interview data will be analyzed thematically. Qualitative and quantitative data will be merged during analysis to arrive at an enhanced understanding of care experiences. Quantitative and qualitative data will be combined and presented narratively using a weaving approach (jointly on a theme-by-theme basis) and visually in a side-by-side joint display.

Ethics and dissemination: The study protocol and procedures were approved by the Children's Hospital of Eastern Ontario (CHEO)'s Research Ethics Board, the University of Ottawa

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Research Ethics Board, and the research ethics boards of each participating study center. Findings will be published in peer-reviewed journals and presented at scientific conferences.

Keywords: Family-centered care, pediatrics, healthcare experiences, inherited metabolic diseases, mixed methods

ARTICLE SUMMARY

Strengths and limitations of this study

- This study will ascertain family perspectives on health care networks and positive and negative care experiences for children with high care needs, such as those with inherited metabolic disease, forming a comprehensive understanding of current care, including gaps in family-centered care that will form the foundation for successful development of complex interventions to improve health care experiences for this understudied population.
- We expect this study to contribute to the methodological literature on assessment of health care experiences by using a novel combination of approaches, including care maps, diaries, and interviews.
- This study exemplifies partnership with patients and their families in co-designing research toward improved health care.
- A limitation of this study is the requirement of English proficiency for study participation, which will exclude a potentially more vulnerable population of children and families who, for example, require language supports for their health care.

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1 BACKGROUND

Inherited metabolic diseases (IMDs) are individually rare genetic conditions, often diagnosed in early childhood, that have a collective estimated global prevalence of 50.9 in 100,000 live births.[1] Many children with IMDs have complex and intensive health care needs.[2,3] Due in part to health service inequities related to infrastructure and funding, they and their families face multiple challenges in receiving high quality care[4] and, in common with children with medical complexities generally, may not receive optimal interdisciplinary family-centered services.[5,6]

Patient experience is a key pillar of a high performing health system.[7–9] Assessments of patient experience frequently address established principles of patient-centered care,[10] including access, coordination and continuity, and communication.[8,11,12] In pediatrics, these principles extend to family-centered care, emphasizing children's developmental needs and recognizing the central role of family members in disease management.[13,14] Families are often experts about the care needs of their children with rare diseases such as IMDs, underscoring the importance of their perspectives and their engagement in both health care and research.[5]

Several studies have focused on the quality of life and caregiving experiences of families of children with IMDs;[15,16,25,17–24] a smaller proportion have identified challenges or needs associated with providing and accessing care.[15–19] To begin to understand the health care experiences of this potentially underserved population, we completed two qualitative studies: first with representatives of relevant patient groups, then with caregivers of children with IMDs enrolled in a Canadian cohort study.[26,27] Overarching themes included a lack of familiarity with IMD care among many care providers outside of the metabolic clinic and poor suitability of some care systems to meet the needs of frequent and complex users. These studies expose a need for interventions that improve health care experiences of children with IMDs and their families. An Australian study found that families of children with IMDs experienced improved health care

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if care was accessed through a coordinating center.[28] Guidance about family-centered care for children with chronic conditions more generally suggests additional potential strategies for addressing some of these challenges, for example, co-developed care plans, receipt of care within a 'medical home', relational continuity with a key provider, improved collaboration between providers, and increased family involvement.[5,13,14,29,30] These potential strategies reflect *complex interventions*: each single strategy would require multiple interacting components, targeting multiple individuals or systems, and customization to specific contexts of care, with potential impacts on a range of outcomes.[31] Guided by the UK Medical Research Council (UKMRC) Complex Interventions Framework,[32,33] we have planned a rigorous, four-phase research program (Supplementary material 1) to develop complex interventions to improve family experiences with care. This protocol outlines our plans for "Phase I", the first study in our research program, in which we seek to build on our previous qualitative studies to more fully understand and describe the 'problem':[26,27,33] the nature, frequency, heterogeneity, and impact of positive and negative health care experiences of children with IMDs and their families. Such a purpose requires both quantitative data that can be generalized to a larger population and qualitative data to understand the nuances of individual experiences and is thus well-suited to a mixed methods design.[34] Mixed methods designs have been used in several studies of patient or family experiences in pediatric health care.[35–42]

1.1 Objectives

This study's overall aim is to comprehensively understand the health care experiences of children with IMDs and their families across Canada.

Quantitative objectives

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- To identify and describe the providers and services included in children's care networks and how they are connected to both the family and to one another, from parents' perspectives
- To prospectively measure the frequency, heterogeneity, and satisfaction with health care encounters of children and their families
- To identify the family characteristics and circumstances that form the context in which families experience health care, and their association with health care encounter satisfaction

Qualitative objectives

- To explain and enhance our understanding of:
    a. parents' perceptions and assessments of their children's care networks
    b. how families experience positive and negative health care encounters

Mixed methods objectives

To merge the quantitative and qualitative findings to arrive at an enhanced understanding of:

- The nature of children's care networks and how they are experienced and assessed by parents
- The family-centered elements and processes related to parent perceptions of positive and negative health care encounters

Pursuit of these objectives will be foundational to understanding how to develop complex, family-centered care interventions. For example, identifying the constellation of providers and services and their roles and connections in children's care networks may enable us to identify key providers for health care coordination interventions (quant, qual). Knowing the most

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frequently-used services will help with the prioritization of intervention development and implementation (quant). Understanding which aspects of care contribute to negative and positive experiences will help inform the creation of responsive interventions (quant, qual). An understanding of family characteristics and situations will shape interventions that account for the challenges and realities faced by families managing their child's care at home (quant).

The COVID-19 pandemic has exacerbated existing challenges related to access to care, and is expected to continue to affect how health care is delivered in the future. Therefore, we will collect data to understand the current context of health care delivery across Canada during the pandemic. In particular, we will aim to understand family experiences with virtual care, since this delivery modality has become more common due to pandemic response measures and the increase in its use is likely to influence health care delivery in a post-pandemic environment.

2 METHODS

2.1 Study design

The UKMRC Complex Interventions Framework, a phased approach to the design, evaluation, and implementation of complex interventions, guided this study's design.[32,33] Following previous studies of health care experiences,[43–47] we will also use the Picker Principles of Patient-Centered Care to provide a framework to guide data collection and analysis regarding key aspects of family-centered care.[12]

We will conduct a mixed methods study, following a two-stage explanatory sequential design (Figure 1).[34] **Stage 1:** Quantitative data will be collected on parent perceptions of children's health care networks (the people involved in a child's health care and how they are connected) and on health care encounters (frequency, context, experiences with care). These data

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will be analyzed on an ongoing basis to inform the sample selection for **Stage 2**: two subsets of participants from Stage 1 will participate in qualitative data collection (interviews) about (i) the participant's perception of the child's care network; and/or (ii) the factors that contributed to a strongly positive or negative health care experience. At the individual level data collection will be sequential: the quantitative collection of data related to the child's care network and experiences will precede the qualitative collection of data related to the network or to a specific health care experience. Data from both stages will be integrated during analysis. We will use the STrengthening the Reporting of OBservational studies in Epidemiology (STROBE) guideline[48] to report the study (Supplementary material 2).

2.2 Patient and public involvement

The interventions informed by this study will be complex, involving diverse systems, providers, and families, and aim to be family-centered. This underscores a need to engage families and providers,[49,50] especially in the context of rare disease where families become experts in their children's care needs.[35] Parents of children with IMD and adults living with IMD are engaged in this study to provide expertise on the family/patient experience. Three family/patient partners (IJ, NP, MS) are study co-investigators, leading the family engagement strategy, advising, and providing expertise, and sharing in decision-making at all study stages, from conceptualization to dissemination. The study also engaged 11 patient/family advisors, recruited through IMD family advocacy and support organizations, to provide advice and feedback during study instrument development; six of them also pilot tested the data collection instruments.

2.3 Quantitative sample

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Participants will be parents or legal guardians ("parents") of children diagnosed with an IMD. Although children's self-report of experiences is important, we seek to understand the experiences of health care for younger children (≤12 years). Parents are the family members most actively involved in seeking and managing health care for their children and thus are likely the best informants to provide comprehensive information on health care for this age group. For each participating family, one parent will be identified by the family as the "designated parent" to provide data regarding one child in their family with an IMD ("designated child").

Eligibility criteria are described in Table 1. Child age will be restricted to ≤12 years as adolescents with chronic conditions have different health care and clinical treatment needs.[51,52] With respect to eligibility of IMD diagnoses, >1000 IMDs have been identified.[53] IMDs typically follow one of three broad clinical course trajectories, with different implications for health care usage and experiences: (a) chronic and generally non-progressive; (b) acute episodes of severe illness with or without accompanying chronic multi-system sequelae; and (c) progressive multi-system disease. Children with any of 30 priority IMDs included in an existing Canadian pediatric cohort study that will serve as one potential recruitment source[54,55] are eligible for this study (Table 1). Few of the IMDs included in that cohort study, however, are characterized as following trajectory (c). Thus, children will also be eligible for this study if they have an IMD that meets clinical criteria associated with trajectory (c) (Table 1), to be evaluated by clinician investigators on a case-by-case basis.

Table 1. Eligibility criteria

Inclusion	Exclusion
• The designated parent and designated child are Canadian residents • The designated child is ≤12 years at pre-screening • The designated child is receiving health care from one of 11 participating pediatric metabolic clinics across Canada: Alberta's Children Hospital, British Columbia Children's Hospital, Children's Hospital of Eastern Ontario, Health Sciences Centre Winnipeg Children's Hospital, The Hospital for Sick Children, IWK Health Centre, Kingston General Hospital, London Health Sciences Centre, McMaster Children's Hospital, Montreal Children's Hospital, Stollery Children's Hospital • The designated child has an IMD that is ***either***	Designated parents who cannot speak, write, and read English comfortably

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1. identified in the following list (these conditions were the focus of an existing cohort study; most have a typical clinical course that aligns with what we call trajectory a or trajectory b):
   - ß-Ketothiolase deficiency
   - Arginase deficiency
   - Argininosuccinic aciduria
   - Carbamoyl phosphate synthetase deficiency
   - Carnitine uptake defect
   - Citrin deficiency
   - Citrullinemia
   - Farber disease
   - Galactosemia
   - Glycogen storage disease type 1
   - Glutaric acidemia type I
   - Guanidinoacetate methyltransferase deficiency
   - HMG-CoA lyase Deficiency
   - Homocystinuria
   - Hyperornithinemia-Hyperammonemia-Homocitrullinuria syndrome
   - Isovaleric acidemia
   - Long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency
   - Maple syrup urine disease
   - Medium chain acyl-CoA dehydrogenase deficiency
   - Methylmalonic acidemias
   - Mucopolysaccharidosis type I
   - Multiple carboxylase/biotinidase deficiency
   - N-acetylglutamate synthetase deficiency
   - Ornithine transcarbamylase deficiency
   - Phenylalanine hydroxylase deficiency
   - Propionic acidemia
   - Pyridoxine-dependent epilepsy
   - Trifunctional protein deficiency
   - Tyrosinemia type I
   - Very long-chain acyl-CoA dehydrogenase deficiency
2. ***or*** meets the following clinical criteria (included to expand the list of eligible conditions and to increase representation of IMDs with a typical clinical course that aligns with what call trajectory c):
   - involves at least three organ systems *and*
   - chronic complications of the disease get progressively worse over time, even with available treatment

In order to collect data on health care experiences from a diverse sample of families, we will use a purposive, maximum variation sampling approach[56–58] to identify and recruit participants. We will aim for maximum variation on six selection variables on which experiences with care are anticipated to vary: study center, travel time from home to study center, child's sex**,** child's age (years), IMD type, and IMD typical clinical course trajectory. Treatment protocols and health care service availability and practice vary by IMD, clinical course classification, study center, and/or distance to specialists.[27,59] Health care encounters tend to be more frequent in the first years following an IMD diagnosis (usually in infancy) and parents characterize this time as uncertain and stressful.[27] Sex differences can affect metabolism, resulting in different care

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experiences for girls and boys.[60,61] We will prioritize the selection of participants who expect the designated child to have ≥1 health care encounter per month during the study to collect sufficient data for analysis.

2.4 Quantitative procedures

Participants will be recruited from the existing cohort study and/or from the study centers across Canada. Eligible parents will be notified of the study by the study team (by telephone) or by their associated study center (by telephone or at a clinic visit). For those notified by telephone, up to three contact attempts will be made. Participants will be enrolled on a rolling basis and the sample continually assessed for diversity on study selection variables to identify characteristics desired for further recruitment. Based on our previous experiences conducting studies with this population, we estimate a 50% response rate. Recruitment commenced in November 2020 and will be concluded when 100 families are enrolled. Interested parents will receive via email a postcard with study information and a link to the online Eligibility and Pre-Screening Questionnaire (5-10 min).

Data collection procedures are outlined in Figure 1. All questionnaires will be web-based. Study data will be collected and managed using Research Electronic Data Capture (REDCap) hosted at the Children's Hospital of Eastern Ontario (CHEO).[62,63] The participant, if they desire, may consult other family members, including the designated child, to complete the data collection tools. Children will continue to access health care normally. Participants will be reminded up to two times to complete each questionnaire.

2.5 Quantitative data elements and instruments

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Data collection instruments are described in Table 2. Care map instructions, sample survey questions and measurements, and interview guides are provided in Supplementary material 3. Instruments were developed with input from clinicians, methodological experts, and family/patient partners and advisors, and pilot tested.

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Table 2. Data collection instruments

Data collection period Data instrument	Data type	Instrument completion time[a] (minutes)	Instrument and data details
Baseline			
Care Map	Quantitative	40	Participant creation of a care map of their perceptions regarding their child's network of care providers, which providers are perceived to work together to coordinate their child's care, and which providers are considered 'key providers' (maximum ten)
Care Map Questionnaire	Quantitative	5	Participant perceptions about: • Coordination of their child's care • Familiarity with their child by identified key health care providers
Baseline Questionnaire	Quantitative	20 – 40	Demographics and potential predictors of health care encounter satisfaction ratings, e.g., child health status, child and family characteristics, family resources in IMD management, and effects of the COVID-19 pandemic on child health and health care since March 2020
Pre-Questionnaire for Weekly Logs	Quantitative	5 – 20	Data will be used to tailor the Health Care Diaries, to reduce repetition of questions where responses are anticipated to remain constant over the study period
Follow-up			
Health Care Diaries[b]	Quantitative, qualitative	5 – 60	Descriptive data on health care encounters including: the mode of interaction, the care setting if applicable, the health care providers involved, the date of the encounter, financial costs, time inputs, and any parent-perceived effects of the COVID-19 pandemic (e.g., on scheduling or delivery of care) Optional, open-ended questions for descriptions of participant perceptions of care in each Picker Principle domain, and for the overall encounter The Experience Questionnaire will be tailored to each encounter's mode of interaction (in-person or virtual/remote), care setting, and context (planned or urgent care; whether it is a 'frequent' care encounter, as identified on the Pre-Questionnaire for the Weekly Logs)
Interviews	Qualitative	30 – 60 30 – 45	a) Map interviews: Seek to understand and elaborate on the care map, including how the participant selected providers to include on the map, the roles and relationships with the family for the providers designated on the map as "key providers", the meaning of connections drawn between providers, and how the participant feels about the effectiveness of the care network, including what improvements they see as potentially important b) Encounter interviews: Seek to clarify, interpret and deepen our understanding of information collected in the Health Care Diaries, specifically: elements of a health care encounter that contributed to participants' high or low satisfaction with that encounter; the impact of these experiences, especially the challenges, on the child, parent, other family members; and the context of general health care for their child (i.e., comparison between this encounter and past similar encounters). Impact will be iteratively defined, depending on the information shared by participants, and may include psychosocial, health, and/or economic impacts.

[a] Estimated

[b] All elements are completed once except the Health Care Diaries, which are completed weekly x 17 weeks

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2.5.1 Care maps

In this study, a 'care map' is a pictorial representation of the networks of health care providers around a child with an IMD and their family, commonly used in research on children with complex or chronic health conditions.[5,64–66] Guided by a set of instructions,[67] care maps will be drawn by hand, photographed, and uploaded to the study data collection database by the participant, and a digital version rendered by the study team.

2.5.2 Baseline questionnaires

Participants will be invited to complete three questionnaires: the Care Map Questionnaire, the Baseline Questionnaire, and the Pre-Questionnaire for Weekly Logs (content overview, Table 2). The Baseline Questionnaire also includes a number of validated instruments. Child health status will be assessed using the Child Health Questionnaire (CHQ-PF50)[68] for children ≥ 5 years or the Infant and Toddler Quality of Life Questionnaire (ITQOL-SF47)[69] for children <5 years. Both are parent-reported measures and have good validity and reliability.[69–71] Parent-perceived quality of life related to caring for the designated child will be measured using the CarerQol instrument. The CarerQol has good psychometric properties[72–75] and has been used with parents of children with chronic conditions, including rare diseases.[76–79] We reformatted the measure for online use.

2.5.3 Health care diaries

The Health Care Diary ("Diary") is composed of two parts: a Health Care Log and Experience Questionnaire. Once per week, participants will record whether a child had any health care encounters in a given week on the Health Care Log. If yes, they will complete an Experience Questionnaire for each of those encounters. Diary methods have been used in health studies to

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capture real-time information to reduce the recall errors associated with retrospective surveys,[80,81] with electronic diaries yielding higher quality data than paper diaries.[82,83] The definition of a health care encounter is provided in Figure 2. Evaluations will be made for the overall experience as well as in eight domains consistent with the Picker Principles of Patient-Centered Care where applicable:[84] access to care, information sharing, care coordination, physical comfort, emotional support, family involvement, respect for the patient/family, and continuity. The Consumer Assessment of Healthcare Providers and Systems Child Hospital Survey,[85] Ontario Emergency Department Patient Experience of Care Survey,[86] Outpatient Survey (Christine Kouri, Manager for Patient Experience, CHEO, e-mail communication, October 2017) and the Cost Utilization Survey for Child Phenylketonuria[87] were used as resources for our diary instrument development; diary questions were either author-developed, informed by, or adapted from these resources.

We will collect prospective data on blood draws done at home by the family, following the same family-centered care domains. For many IMDs, blood draws are essential to the ongoing monitoring of a child's health status, and though sometimes conducted by the family, require an ongoing dialogue with health care providers to adjust a child's medication, diet, or other treatment.

2.6 Qualitative sample

The two qualitative samples will be nested in the quantitative sample. Qualitative participants queried about their children's care networks must have completed the Care Map Questionnaire, and those queried about their positive or negative encounters must have completed at least four diaries. For the interview focused on the health care encounter ("encounter interview"), we will select participants who have had a health care encounter with which they reported they were

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"extremely satisfied", "extremely dissatisfied", or "somewhat dissatisfied" overall or on at least one family-centered care domain. We will use purposive, maximum variation sampling and extreme case sampling to separately sample participants for each interview set,[56–58] aiming for sample variation across the selection variables used for the quantitative sample and across health care settings in the encounter interviews. For the encounter interviews, if the parent who accompanied the child to the encounter is not the designated parent, they will be invited but asked to provide informed consent before proceeding. Some participants in the quantitative sample may be invited to participate in both interviews.

2.7 Qualitative procedures and data elements

On a rolling basis, participants will be identified and invited by e-mail to participate in a one-on-one, semi-structured interview held by videoconference or by audioconference, according to participant preference. For the interview focused on care network ("map interviews"), participants may be sampled at any time after completing the Care Map Questionnaire. For the encounter interviews, participants will be sampled during and up to three weeks after completing week 17 of the Diaries. Interviews will be audio-recorded with participant consent and transcribed. Up to three attempts to contact participants will be made to invite interview participation. Both interview sets will be semi-structured and informed by an interview guide.

2.8 Sample size

While we did not conduct a formal power calculation for the quantitative part of this study, given our largely descriptive purpose, we deemed a sample size of 100 families sufficiently large to support planned analyses across a heterogeneous sample, while maintaining feasibility for recruitment and study administration.

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Because of the duration and intensity of study participation, we anticipate some dropout. Dropout rates may increase with longer study lengths.[88,89] To facilitate participant retention, we pilot tested the feasibility of study questionnaires. In addition, we will: 1) enroll a new participant to replace any participant withdrawn before completion of at least four Diaries; 2) actively monitor completion of study instruments and follow up with participants if necessary; 3) provide participants with financial compensation (up to $400 in gift cards) for their time and as a participation incentive;[90] 4) encourage the scheduling of time each week to complete the Diaries; 5) allow for instrument completion over multiple sittings; and, 6) allow for flexibility of instrument completion.

A participant will be considered lost to follow-up upon notification of withdrawal or non-completion of an instrument within pre-specified timeframes; they will have the option to continue in the study if they proactively express a desire to do so. Data collected up to time of withdrawal will be included in the study.

The qualitative sample sizes will not be determined in advance; they will be assessed continuously and finalized during data collection. Information power is a methodological model for determining a qualitative sample size, and has five contributing dimensions related to: narrow vs broad qualitative objectives; the homogeneity of the sample on important characteristics; use of a theoretical framework; quality of interview data; and planned analytic strategy (case vs cross-case analysis).[91] Based on this concept and previous qualitative studies with parents of children with chronic conditions,[26,92–94] we anticipate a sample size of approximately 15-30 participants for each interview set.

2.9 Analyses

2.9.1 Quantitative analyses

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We will describe continuous variables using means and standard deviations or medians and interquartile ranges, and categorical variables using counts and proportions (%). Baseline data will be analyzed to describe the characteristics of participating families, including child and parent demographic variables, quality of life, experiences with managing an IMD in the context of COVID-19, and experiences with managing an IMD in general, including time and cost impacts.

From the care maps, children's networks of care providers and their interactions will be analyzed using an adapted form of social network analysis,[95,96] conducted using UCINET software.[97] We will describe who is in the network (nodes), identify the most common providers perceived as key providers, and analyze connections among providers from parents' perspectives (social network analysis calculations of network size and density and the degree centrality of providers).

From the Diaries, we will calculate the frequency (count and rate) of encounters by participant/child, accounting for follow-up time contributed. We will calculate counts and proportions to describe characteristics of health care encounters (e.g. pre-planned vs unplanned, the types of health care providers interacted with, care setting or mode of interaction), overall satisfaction ratings, and satisfaction ratings by Picker Principles (access to care, communication, coordination of care, etc).

To explore the potential relationships between a range of explanatory variables and satisfaction with health care experiences, depending on data quantity and distribution, we propose to use generalized linear regression analysis. The unit of analysis will be the individual health care encounter with each child having potentially different numbers of encounters. Explanatory variables will include both time-fixed and time-varying factors, namely child, family, and setting/provider characteristics (e.g., child age, IMD clinical course trajectory, travel

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time from home to care setting, socioeconomic status), health care setting, and mode of interaction. The five-point ordinal score for the overall experience of the health care encounter will be analyzed using ordinal logistic regression. Correlation in repeated measures on the same child will be accommodated either by directly modeling the covariance matrix or through the addition of child-specific random effects. A similar approach will be used to analyze the experience ratings within the eight family-centered care domains.

This study will minimize missing data by regularly monitoring completion of instruments and diary entries and following up with participants as necessary. Participants will have access to ongoing support from the study team. We will report on the number of missing values for each variable of interest, the reasons for missing values (if known), characteristics of participants with missing vs non-missing values for key variables, and missing data counts for each analysis. Our analytic strategy for managing missing data will depend on the extent of missingness of data for particular analyses and may rely on complete case analysis or multiple imputation. Withdrawn participants will be considered lost to follow-up at the date of their last completed baseline instrument or Health Care Diary.

2.9.2 Qualitative analyses

Guided by principles of family-centered care but also incorporating an inductive approach, we will use thematic analysis[98] to guide the coding and analysis of qualitative data across participants, using the following recommended process: 1) Review the interview transcripts and familiarize themselves with the data; 2) Do an initial, systematic coding of the data; 3) Identify themes of codes; 4) Review the generated themes against both the initial codes and the original data; 5) Refine the themes; and 6) Select and review extracts to illustrate the themes. We will

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repeatedly cycle through steps, particularly steps 3-4, to ensure the themes remain reflective of the original data.[98]

2.9.3 Mixed methods integration and analysis

The two types of data will be integrated at several points in the study. The quantitative data will be used to inform the qualitative sample as well as the interview questions and topics. We will compare the qualitative sample to the quantitative sample on the quantitative sampling selection variables. The quantitative and qualitative results will be merged in analysis and integrated to better understand the elements and processes related to health care networks and to positive or adverse health care encounters than would be gained from either data type alone.[34] In the final report, the qualitative and quantitative results will be integrated narratively using a weaving approach[99] (reported together, grouped by theme or concept) and presented visually in a side-by-side joint display with interpretations of the combined results and inferences about the meaning of the integrated data.[100]

3 DISCUSSION

This study will collect important information about parent perceptions about their families' experiences with health care for children with IMDs, a population with complex needs. Few pediatric studies have attempted to collect similarly comprehensive data on health care experiences.[41] Previous studies of children with IMDs and their families have focused on the impact of the IMD on the child, caregiver, or family well-being;[16–22,25] and/or on family experiences managing health care.[15,23,25] To our knowledge, this is the first broad study of health care experiences in pediatric IMD. We have designed a study that draws on mixed methods that best suit the research objectives, enabling the collection of experiential information

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of both breadth and depth. Diaries are an innovative tool in health research with potential for collecting real-time quantitative and qualitative data simultaneously. Care maps provide useful insight on how participants conceive of the networks of care around their children.

The main findings of this study will inform future phases of our research program, culminating in the co-development of family-centered interventions to improve health care for children with IMDs and their families. Comprehensive, prospective information collected on individual health care experiences will help elucidate the elements of health care that contribute to caregivers' negative and positive experiences. This information will also enable an assessment of the degree to which health care experiences are family-centered, ultimately helping to inform the creation of responsive interventions, especially for highly-frequented services. Care map data will identify key providers and enable an understanding of how participants perceive providers to be connected to each other and to the family. This may help to identify key providers who may be able to lead a child's 'medical home', playing an active role in coordinating their health care. Knowledge about the time, financial costs, and other inputs required to care for a child with an IMD is necessary to ensure that interventions are responsive to the realities of families for whom the interventions are designed to support. Data captured on health care experiences during the COVID-19 pandemic will contribute important information on the benefits and drawbacks of significant changes to health care delivery, such as virtual health care, that can improve the way that this care is delivered in the future. Through our larger research program, the evidence generated in this study will have a direct, actionable impact on family-centered health care for pediatric IMDs.

This study has limitations. All study data will be sought from parents. Their perceptions of their child's health care, for example, whether or not two providers work together to coordinate their child's care, may differ from providers' perceptions. However, health care providers will be

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interviewed about their perceived barriers to and facilitators of effective health care for children with IMDs in the next phase of the research program. Requiring English proficiency for study participation will limit the generalizability of study findings and will exclude a potentially more vulnerable population of children and families who, for example, require access to translators and additional supports as part of their care.

This study may be affected by selection and information biases. We will prioritize the selection of participants who expect the designated child to have multiple health care encounters during the study; our quantitative sample will be over-representative of families who are frequent health care users. This characteristic of our anticipated sample will increase the number of prospective health care experiences reported; however, it may limit the generalizability of quantitative findings. Although Canada has a publicly-funded health care system, access to all care and services is not equitable[101]. A higher frequency of encounters may indicate greater access to care. Children with fewer expected encounters will still be enrolled in the study, and access to care (unavailable services, out-of-pocket expenses) will be analyzed. Past positive or negative experiences with care may motivate parents to participate in a study that provides the opportunity to share those problems and experiences. Non-response bias has been associated with both high and low patient satisfaction.[102,103] Parents whose children are experiencing urgent or critical health care issues, whose children are newly diagnosed (often associated with younger age), or who experience significant financial and time costs may feel overwhelmed and be less likely to participate or remain in the study than parents whose children's health issues are relatively stable.[26,104] We will attempt to minimize the burden of study participation by employing web-based data collection and offering compensation for study participation. To ensure that lack of home Internet access is not a barrier to study participation, participants may

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be loaned a study tablet with a mobile data plan to participate in the study. We anticipate that this may affect 10 - 15 participants.[105]

Factors such as recall and negativity bias may affect the reporting of all health care encounters. Our collection of prospective data via diaries, however, aims to capture experiences during all health care encounters, positive and negative, with a high frequency of reporting to mitigate errors associated with recall time.[81,106] The perspectives of the interviewer and data analysts may affect the collection and analysis of qualitative data. Interviewers will be trained by investigators with expertise in qualitative interviewing. Interviews will be transcribed as soon as possible after interviews and reviewed.

4 ETHICS AND DISSEMINATION

The study protocol and procedures were approved by associated research ethics boards (Supplementary Material 4). Participants will provide informed consent. Study data will be analyzed and stored securely.

Study findings will be published in peer-reviewed, open access journals and presented at relevant conferences. Additionally, a summary of study results will be shared with interested participants (opt-in). Study results will also inform future phases of our research to develop interventions to improve family-centered health care for this population.

FIGURES

Figure 1. Study design overview: mixed methods explanatory sequential design

Figure 2. Health care encounter definitions/eligibility

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SUPPLEMENTARY MATERIALS

Supplementary material 1. Research program overview. Figure illustrating the overall research program and contextualizing this study (Study 1) within it

Supplementary material 2. Completed STROBE checklist

Supplementary material 3. Summary of study questionnaires and instruments. List of study questionnaires with sample questions and copyrighted instruments used, care map instructions, and interview guides

Supplementary material 4. Research ethics committee approvals. List of research ethics committees that have approved this study.

DECLARATIONS

Availability of data and materials

Not applicable

Competing interests

SD has been or is a member of advisory boards for, received indirect educational grants from, and/or received indirect speakers' fees from Sanofi-Genzyme, Takeda, and Horizon Therapeutics. IDG is a recipient of a CIHR Foundation Grant (FDN# 143237). MP has been an advisory board member with honoraria from Ultragenyx (Mar/Apr 2021) and Horizon Therapeutics (Oct 2020), and received a speakers' honorarium (Sep 2020) and small investigator grant ($6,000, 2019) from Horizon Therapeutics. SS received educational grants from Biomarin, Shire, Recordati and serves/served as PI in clinical trials and postmarketing registries sponsored by Actelion, Biomarin, Shire, Ultragenyx. KW is the CEO of CANImmunize Inc. The remaining authors declare that they have no competing interests.

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Author contributions

BKP, PC, JB, EC, SD, LJG, CRG, JMG, RH, AJ, IJ, SK, JJMacKenzie, NM, JJMitchell, SGN, NP, AS, MS, KNS, RS, SS, MTeitelbaum, YT, CVK, JSW, BJW and KW conceived the study. AJC, BKP, RI, ML, KT, IJ, MS, NP, ZA, PC, JB, EC, IDG, CRG, SG, JMG, RH, AJ, SK, NM, JJMitchell, SGN, AP, MP, CP, LAP, AS, MTaljaard, MTeitelbaum, RS, SS, YT, and BJW designed and planned the study. AJC and BKP drafted the manuscript. AJC, BKP, RI, ML, KT, IJ, NP, MS, ZA, PC, JB, AC, EC, SD, LJG, SG, IDG, CRG, JMG, RH, SJG, AJ, SK, JJMacKenzie, NM, JJMitchell, SGN, AP, MP, CP, LAP, AS, KS, RS, KNS, SS, MTaljaard, MTeitelbaum, YT, CVK, JSW, BJW and KW reviewed/revised the manuscript. All authors approved the final manuscript.

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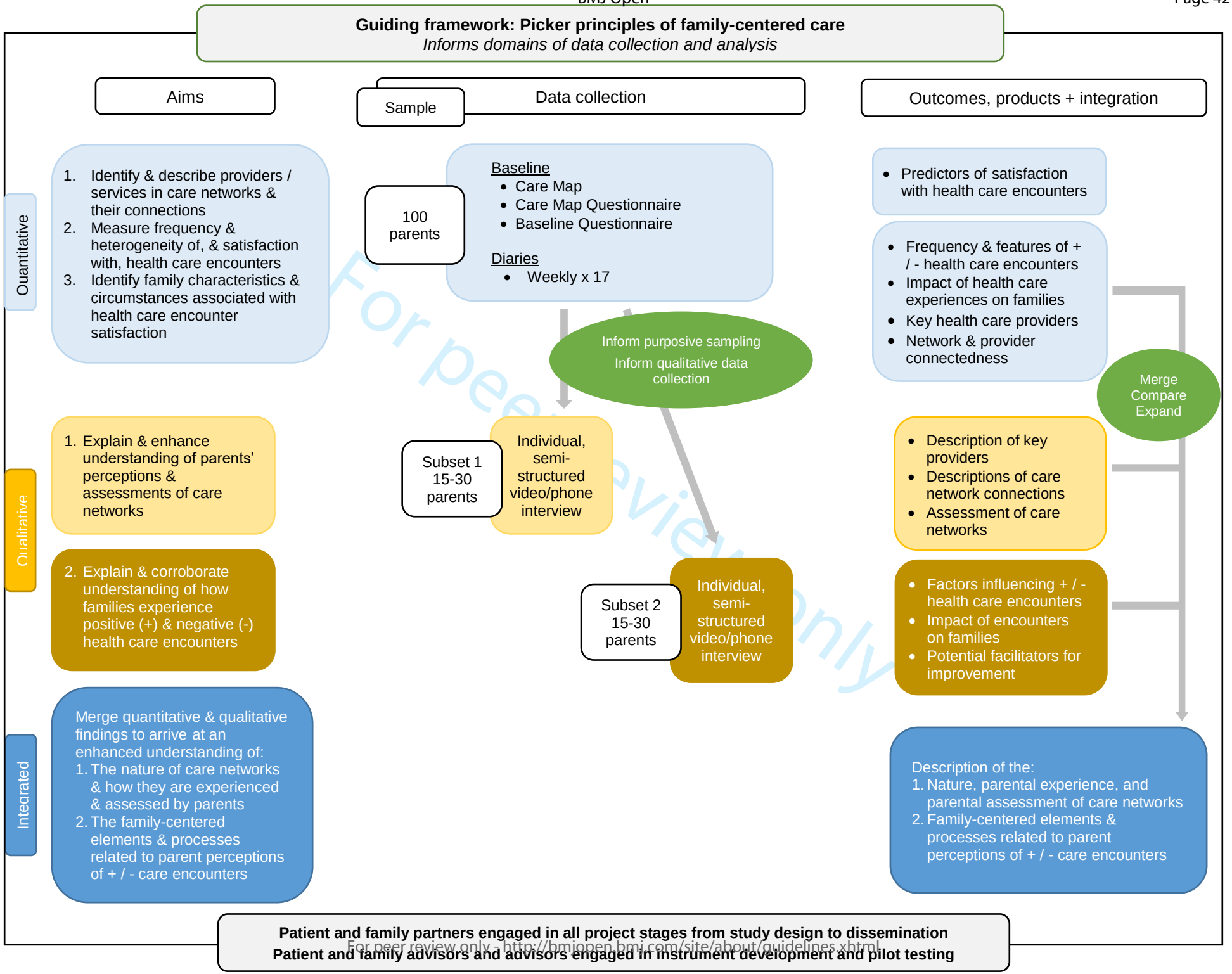

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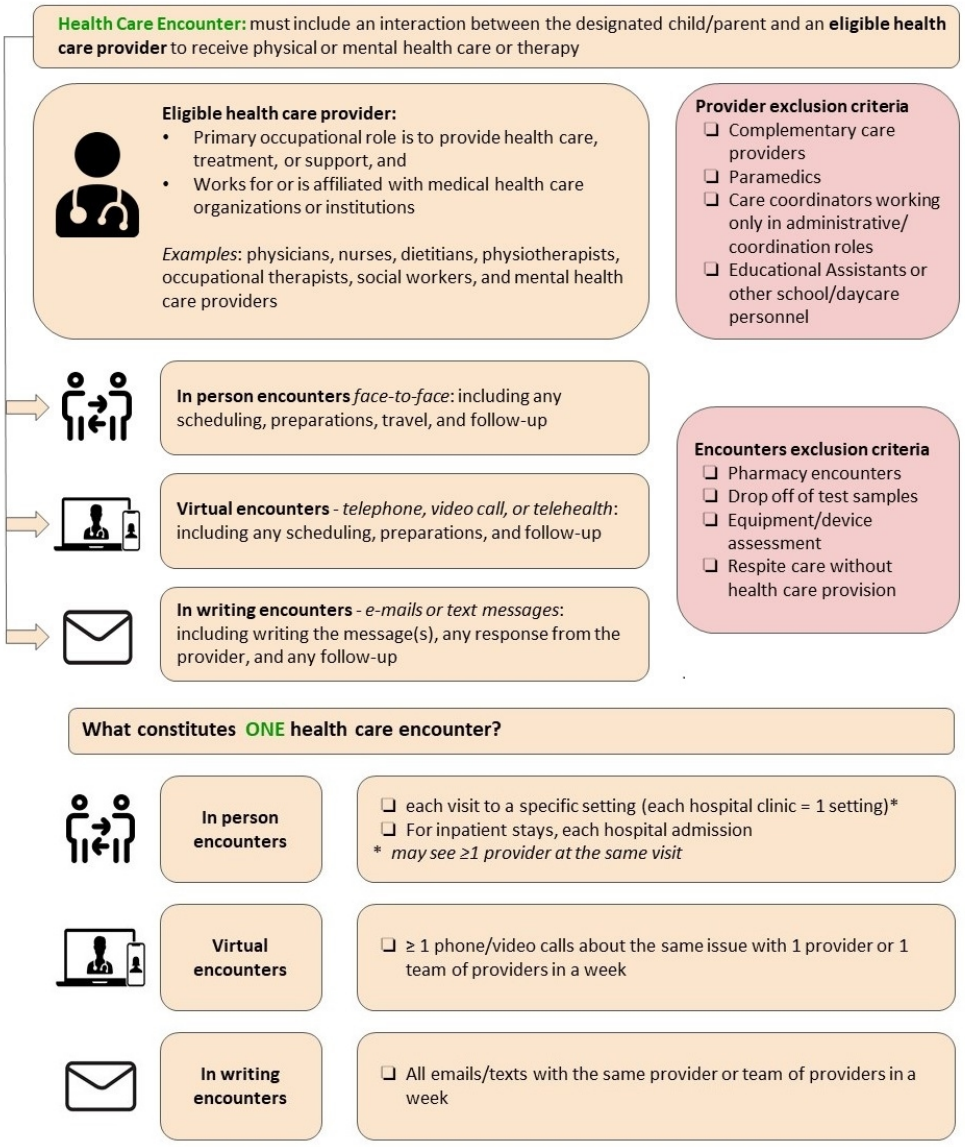

Figure 2. Health care encounter definitions/eligibility

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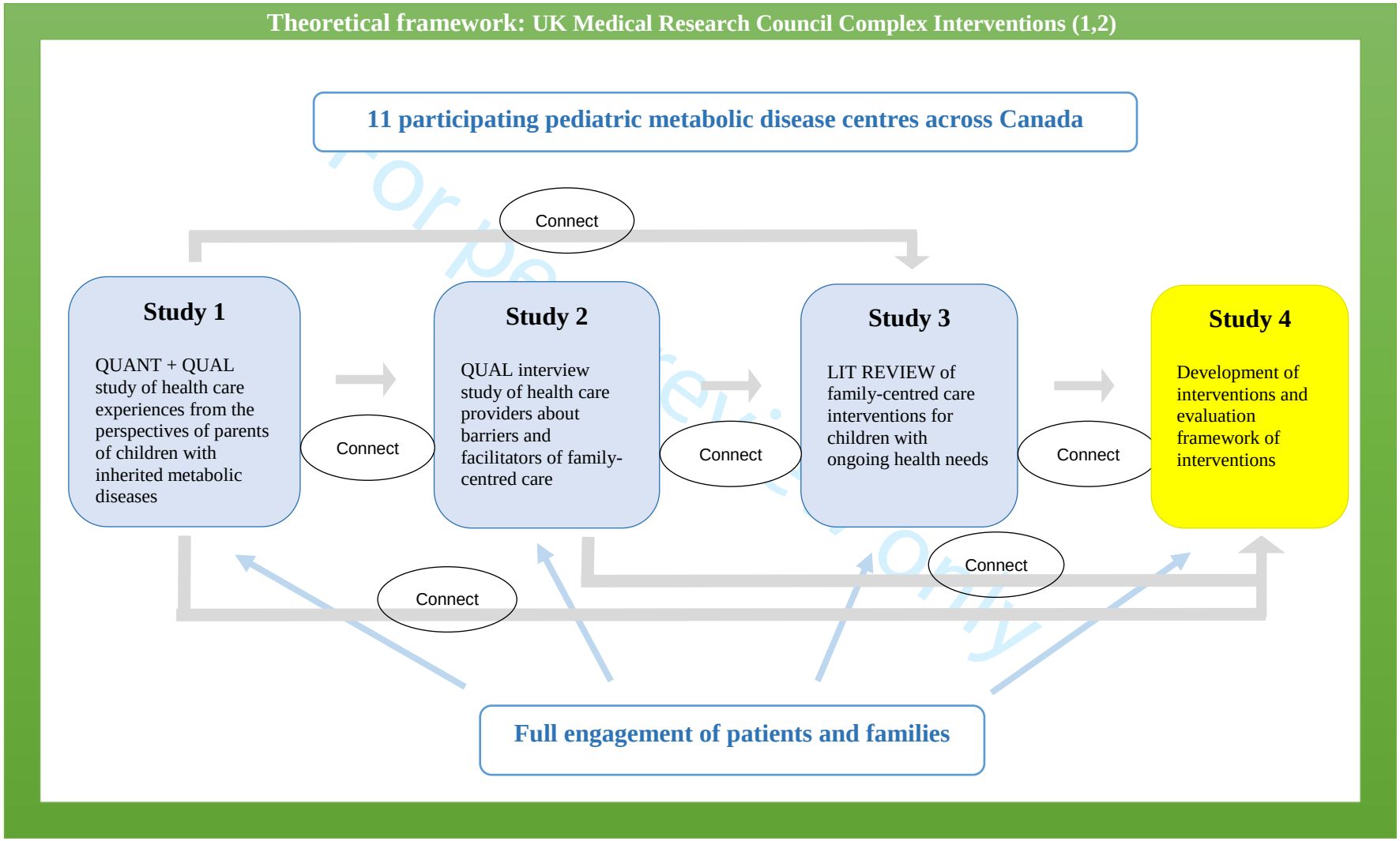
Supplementary material 1
Research Program Overview



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Supplementary material 2 – Completed STROBE checklist

	Item No	Recommendation	Page No
Title and abstract	1	(*a*) Indicate the study's design with a commonly used term in the title or the abstract	1
		(*b*) Provide in the abstract an informative and balanced summary of what was done and what was found	4
Introduction			
Background/rationale	2	Explain the scientific background and rationale for the investigation being reported	5 - 9
Objectives	3	State specific objectives, including any prespecified hypotheses	7 – 8
Methods			
Study design	4	Present key elements of study design early in the paper	9 – 10
Setting	5	Describe the setting, locations, and relevant dates, including periods of recruitment, exposure, follow-up, and data collection	13
Participants	6	(*a*) *Cohort study*—Give the eligibility criteria, and the sources and methods of selection of participants. Describe methods of follow-up *Case-control study*—Give the eligibility criteria, and the sources and methods of case ascertainment and control selection. Give the rationale for the choice of cases and controls *Cross-sectional study*—Give the eligibility criteria, and the sources and methods of selection of participants	10 – 13, 16 – 18, Figure 1
		(*b*) *Cohort study*—For matched studies, give matching criteria and number of exposed and unexposed *Case-control study*—For matched studies, give matching criteria and the number of controls per case	n/a
Variables	7	Clearly define all outcomes, exposures, predictors, potential confounders, and effect modifiers. Give diagnostic criteria, if applicable	13 – 16
Data sources/ measurement	8*	For each variable of interest, give sources of data and details of methods of assessment (measurement). Describe comparability of assessment methods if there is more than one group	13 – 17
Bias	9	Describe any efforts to address potential sources of bias	22 – 24
Study size	10	Explain how the study size was arrived at	17 – 18
Quantitative variables	11	Explain how quantitative variables were handled in the analyses. If applicable, describe which groupings were chosen and why	n/a
Statistical methods	12	(*a*) Describe all statistical methods, including those used to control for confounding	18 – 21
		(*b*) Describe any methods used to examine subgroups and interactions	19 – 20
		(*c*) Explain how missing data were addressed	20
		(*d*) *Cohort study*—If applicable, explain how loss to follow-up was addressed *Case-control study*—If applicable, explain how matching of cases and controls was addressed *Cross-sectional study*—If applicable, describe analytical methods taking account of sampling strategy	n/a
		(*e*) Describe any sensitivity analyses	n/a

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Results			
Participants	13*	(a) Report numbers of individuals at each stage of study—eg numbers potentially eligible, examined for eligibility, confirmed eligible, included in the study, completing follow-up, and analysed	n/a
		(b) Give reasons for non-participation at each stage	n/a
		(c) Consider use of a flow diagram	n/a
Descriptive data	14*	(a) Give characteristics of study participants (eg demographic, clinical, social) and information on exposures and potential confounders	n/a
		(b) Indicate number of participants with missing data for each variable of interest	n/a
		(c) *Cohort study*—Summarise follow-up time (eg, average and total amount)	n/a
Outcome data	15*	*Cohort study*—Report numbers of outcome events or summary measures over time	n/a
		Case-control study—Report numbers in each exposure category, or summary measures of exposure	n/a
		Cross-sectional study—Report numbers of outcome events or summary measures	n/a
Main results	16	(*a*) Give unadjusted estimates and, if applicable, confounder-adjusted estimates and their precision (eg, 95% confidence interval). Make clear which confounders were adjusted for and why they were included	n/a
		(*b*) Report category boundaries when continuous variables were categorized	n/a
		(*c*) If relevant, consider translating estimates of relative risk into absolute risk for a meaningful time period	n/a
Other analyses	17	Report other analyses done—eg analyses of subgroups and interactions, and sensitivity analyses	n/a
Discussion			
Key results	18	Summarise key results with reference to study objectives	n/a
Limitations	19	Discuss limitations of the study, taking into account sources of potential bias or imprecision. Discuss both direction and magnitude of any potential bias	22 – 24
Interpretation	20	Give a cautious overall interpretation of results considering objectives, limitations, multiplicity of analyses, results from similar studies, and other relevant evidence	n/a
Generalisability	21	Discuss the generalisability (external validity) of the study results	n/a
Other information			
Funding	22	Give the source of funding and the role of the funders for the present study and, if applicable, for the original study on which the present article is based	25

*Give information separately for cases and controls in case-control studies and, if applicable, for exposed and unexposed groups in cohort and cross-sectional studies.

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Supplementary material 3 - Summary of study questionnaires and instruments

A. Care map instructions

Making Your Child's Care Map

What is a care map?

A care map shows the people involved in your child's health care and how each person is connected to your child and to each other. An **example** is on **page 3.**

How to make your child's care map

The care map should reflect **how you see** your child's care, who's involved and how they're connected. There is no one way to create a care map. You can draw your own or use the template on page 4. It's up to you. Don't worry about getting it 100% right. If you would like, your child can help you draw the care map.

Things to remember

1. **Please do not put your child or other names on the care map.** Use "child," "family" and job titles instead.

2. When adding people or organizations that are part of your child's health care team. Group them together in a way that makes sense to you. See page 5 for examples of people and organizations that you could include. But there can be others!

3. **Try to include the people involved in your child's health care, not just organizations** (e.g. add teacher, Education Assistant, etc instead of just "school").

What if my child sees 2 people with the same job title in the same clinic?

1) Label them Job Title #1, Job Title #2, etc.

2) Decide whether you consider one of them to be the **main "job title".** If yes, put a star next to Job Title #1. Example: if your child sees 2 metabolic physicians at the metabolic clinic, Dr. Chan, the one your child usually sees, and Dr. Singh, the one you see if Dr. Chan is away, label as follows: "Metabolic Physician #1★" and "Metabolic Physician #2". See Figure 1 below.

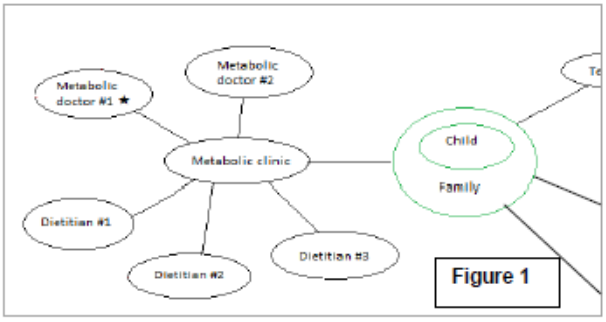

Figure 1

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4. **Connect providers:** Add lines to connect people or groups who work together for your child's health care, for example, by sharing information, providing or receiving referrals. People can be connected to others in same group or organization or at different groups. (See example, Page 3.)

> What if I don't know if 2 people work together or not?
>
> That's OK. Just draw the connections that you know about.

5. **IMPORTANT: Identify up to 10 key providers:** On the Care Map, put the letters "**KP**" next to that person's job title. **Key provider** = someone you think is key to your child's health care. If you do not think any of your child's caregivers is a key provider, just write "No key providers." (See example, Page 3.)

Once you are finished the care map

1. **Take a picture** of the care map or **save as a PDF** file. Make sure it is readable in the image.

2. To **upload the picture,** follow the steps in the email we sent you with this document. Please do not email the picture to the study team.

3. We will make a digital version of your care map. We will send you a link to view it and make sure that it is correct.

Questions?

If you have any questions while creating or uploading your care map, please **contact Andrea Chow**, study coordinator, at **(613) 562-5800 x4353**, or by email at **achow@uottawa.ca**.

Instructions adapted from: Antonelli, RC and Lind, C. Care Mapping: A How-To Guide for Patients and Families. http://www.childrenshospital.org/-/media/Care-Coordination/CareMappingforfamilies21813.ashx?la=en&hash=D8C02FCA893C9A29C939613532334E07127BF9E6. Accessed September 8, 2017.

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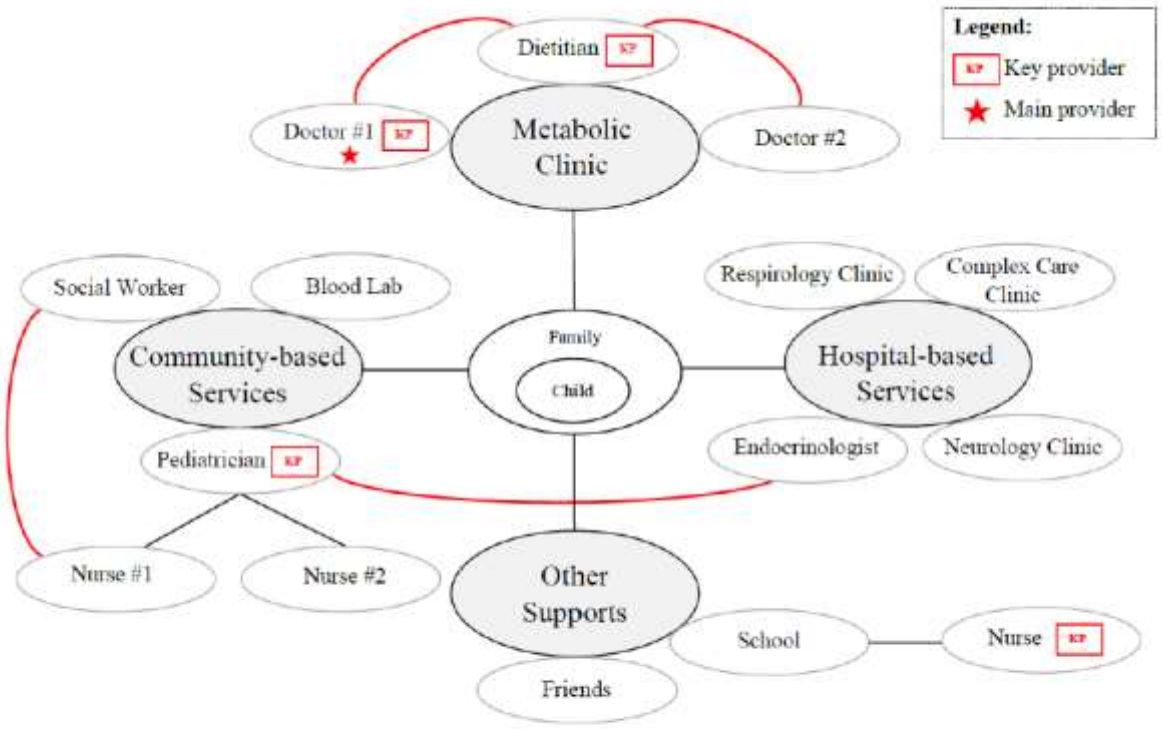
Care Map Example



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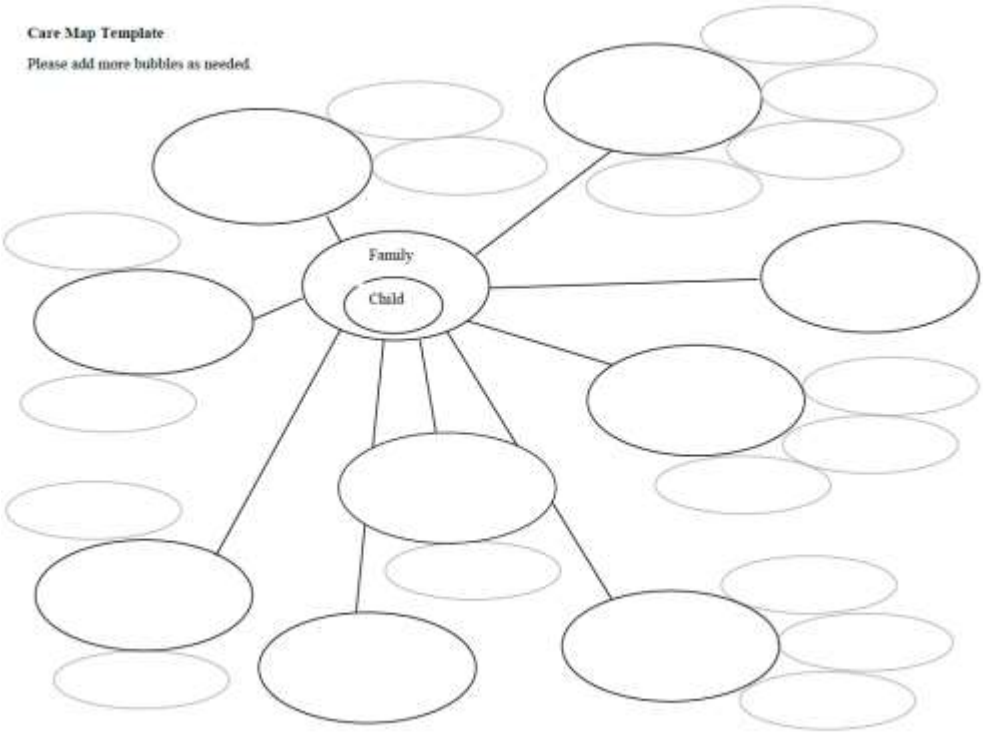
Care Map Template

Please add more bubbles as needed



EXAMPLES

SETTINGS – IN HOSPITAL

Specialty Clinics
Audiology or Speech Therapy Clinic
Cardiology Clinic
Complex Care Clinic
Dental Clinic
Dermatology Clinic
Ear Nose Throat Clinic
Endocrinology Clinic
Gastroenterology Clinic
Hematology Clinic
Metabolic Clinic
Nephrology Clinic
Neurology Clinic
Optometry / Ophthalmology Clinic
Orthodontics Clinic
Orthopedic Clinic
Pediatrician's Clinic
Physiotherapy Clinic
Psychology / Psychiatry Clinic
Rehabilitation Clinic
Respirology Clinic
Rheumatology Clinic
Urology Clinic

Other
Ambulatory or Day Unit
Emergency Department
Feeding or Nutrition Clinic
Genetics Unit
ICU
Inpatient Unit
Laboratory
Mental Health / Counselling Services
NICU
Palliative Care Unit
Urologist
Radiology Unit

SETTINGS – IN HOSPITAL CONTINUED

Sleep Clinic
Social Work Unit

SETTINGS - IN COMMUNITY

Blood Lab
Clinic
Community Centre
Daycare
Diagnostic Imaging or other Laboratory
Hospice
Primary Health Care Clinic
School
Sleep Clinic
Walk-in or Urgent Care Clinic
Your Home

JOB TITLES

Acupuncturist
Audiologist
Behavioural therapist
Cardiologist
Care coordinator
Chiropractor
Complex care doctor
Counsellor
Critical care doctor
Dentist
Dermatologist
Dietitian
Doctor
Ear nose throat doctor
Educational Assistant
Endocrinologist
Family doctor

JOB TITLES CONTINUED

Gastroenterologist
Genetic counsellor
Geneticist
Hematologist
Homeopath
Lab technician
Massage therapist
Mental health professional
Metabolic doctor
Naturopathic doctor
Nephrologist
Neurologist
Nurse
Nurse practitioner
Occupational therapist
Ophthalmologist
Optometrist
Orthodontist
Orthopaedic doctor
Palliative care doctor
Paramedic
Personal support worker
Pediatrician
Pharmacist
Pharmacy assistant
Pharmacy technician
Physical therapist
Physiotherapist
Psychiatrist
Psychologist
Respirologist
Rheumatologist
Social worker
Speech therapist
Surgeon
Therapist

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B. Care map questionnaire

For each key provider identified on the Care Map, the following two questions are asked:

Question	Response options
How well does each of your child's key Health Care Providers know your child?	5-point Likert type scale
How well do you think your child's key health care providers coordinate your child's care with other providers?	5-point Likert type scale

C. Baseline questionnaire

Participants first complete either the **Child Health Questionnaire (CHQ-PF-50)** (if child age ≥ 5) or the **Infant Toddler Quality of Life Questionnaire (ITQOL-SF-47),** followed by author-developed questions, below:

Question	Response options
Your participating child	
What type of inherited metabolic disease does your child have?	Select one from list
What sex was your child assigned at birth?	Select one from list
In which province or territory does your family live?	Select one from list
At which metabolic clinic does your child currently receive the most care?	Select one from list
Does your participating child have any OTHER chronic illness or special needs?	Yes / No
Yes: To what extent do your participating child's special needs and/or chronic illnesses NEGATIVELY affect your family's emotional well-being?	Select one from list
Has your participating child had a major medical event or health crisis in the past two months?	Yes / No
Your child's caregivers	
INCLUDING YOU, how many primary caregivers does the participating child have?	Select # from list
For each caregiver:	
What is your relationship to your participating child?	Select one from list
What gender do you identify with most?	Select one from list
What is the highest level of education that you have COMPLETED?	Select one from list
What is your CURRENT employment status for paid work?	Select one from list
Are you or have you ever been a landed immigrant, permanent resident, or refugee to Canada?	Yes / No
Yes: In what YEAR did you first become a permanent resident, landed immigrant, or obtain refugee status in Canada?	Year
Participant completes the **Carer QOL-7D**	
Does your child have any secondary, UNPAID caregivers?	Yes / No
Yes: How many secondary UNPAID caregivers does your child have?	Select # from list
Does your child have any PAID caregivers?	Yes / No
Yes: How many paid caregivers does your child have?	Select # from list
Other members of your household	
Besides your participating child, how many children under the age of 18 live in your household ALL or SOME of the time?	Select # from list
1 or more: Besides your participating child, how many of the other 2 children in your household have the same inherited metabolic disease as your participating child?	Select # from list
Besides your participating child, do any of the OTHER [#] children in your household have any other chronic illness or special needs?	Yes / No
Yes: How much do the special needs and/or chronic illnesses of your OTHER child(ren) affect your family's resources (physical, financial, time, emotional, etc)?	Select one from list

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Besides your participating child, have any of your OTHER [#] child(ren) had a major medical event or health crisis in the past two months?	Yes / No
Do any of your [#] other children help to care for your participating child?	Yes / No
Yes: How many of the other # children in your household help to care for your participating child?	Select # from list
Managing the Inherited Metabolic Disease	
COVID-19 has changed the way that health care is provided. In general, how do you feel about the quality of your child's health care since the start of the pandemic (i.e., March 2020)?	5-point Likert type scale
Has your child been diagnosed with COVID-19?	Yes / No
Yes: When did they receive the diagnosis?	Month + year
Since then, have they needed extra health care because of their COVID-19 diagnosis?	Yes / No
Right now, do they still need extra health care because of their COVID-19 diagnosis?	Yes / No
How much do you agree/disagree with each statement for your child? Because of my child's COVID-19 diagnosis...	Matrix
...My child's well-being is worse	5-point Likert type scale
...My well-being is worse	5-point Likert type scale
...The well-being of other family member(s) besides me/my child is worse	5-point Likert type scale
Has anyone else in your family been diagnosed with COVID-19?	Yes / No
Over the past 6 months, how have the changes to health care and other services due to the pandemic affected your child's health care? Because of the pandemic...	Check all that apply: 1, One or more of my child's health care appointments or services were cancelled | 2, One or more of my child's health care appointments or services were delayed | 3, One or more of my child's health care appointments were changed from in-person to virtual (e.g., phone, video) | 4, I could not get to the lab, test centre, or pharmacy because their opening hours were reduced | 5, Only one primary caregiver was allowed to go with my child to a health care encounter | 0, None of the above
Checked any 1 – 5: Over the past 6 months, how have these changes to health care services due to the pandemic affected your child's CURRENT health or well-being? Because of the pandemic....	Check all that apply
Checked 1: What services or therapies were cancelled?	Check all that apply
Checked 2: What services or therapies were delayed?	Check all that apply
Checked 3: Overall, how did the virtual appointment(s) compare to similar in-person appointments before the pandemic (i.e., March 2020)?	5-point Likert type scale
Checked 3: Compared to similar in-person appointments before the pandemic (i.e., March 2020)...	Matrix
...the virtual appointment(s) were _____.	Shorter | the same | longer
...on the day of the virtual appointment(s), the wait for the provider was usually _____.	Shorter | the same | longer
...scheduling the virtual appointment(s) was _____.	Easier | the same | harder
...communicating with the provider during the virtual appointment(s) was _____.	Easier | the same | harder
...keeping my child comfortable during the virtual appointment(s) was _____.	Easier | the same | harder
...understanding what steps would take place after the virtual appointment(s) was _____.	Easier | the same | harder

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Checked 3: How was your privacy during the virtual appointment(s), compared to similar in-person appointments before the pandemic (i.e., March 2020)?	Select one from list
Checked 3: Did you feel more or less involved in decision-making about your child's health during the virtual appointment(s), compared to similar in-person appointments before the pandemic (i.e., March 2020)?	Select one from list
Checked 3: Compared to similar in-person encounters before the pandemic (i.e., March 2020), how much do you agree with the following statements?	Matrix
It was convenient to avoid travelling.	5-point Likert type scale
We were able talk to more than 1 provider at the same time.	5-point Likert type scale
The treatment was less effective.	5-point Likert type scale
It cost us less (out-of-pocket costs).	5-point Likert type scale
Checked 3: If the virtual appointment(s) were different in other ways compared to in-person appointments that took place before the pandemic (i.e., March 2020), please describe in the space below.	Open text
How much do you agree/disagree with each statement for your child? Because of the pandemic...	Matrix
...I avoided bringing my child to the emergency department or other parts of the hospital for treatment or care	5-point Likert type scale
...I avoided bringing my child to our primary care provider for treatment or care	5-point Likert type scale
...I had a hard time getting my child's medication or medical products	5-point Likert type scale
Because of the pandemic, I do not want my child to have in-person medical appointments	5-point Likert type scale
I worry about my child getting COVID-19	5-point Likert type scale
Compared to other children, my child is more at risk for COVID-19 complications because of their IMD	5-point Likert type scale
During the pandemic, I have taken public transportation or shared car services to take my child to in-person medical appointments. This has caused me stress or anxiety	5-point Likert type scale
During the pandemic, getting other health care-related needs for my child (e.g., supplies, medication) has caused me stress or anxiety	5-point Likert type scale
During the pandemic, managing my child's IMD at home has been more difficult	5-point Likert type scale
Since the start of the pandemic (i.e., March 2020), how has the pandemic affected your family?	Check all that apply
Do you have a plan, protocol or written directions from the metabolic clinic for managing your participating child's metabolic disease (e.g. a sick day protocol)?	Yes / no
OVER THE PAST 12 MONTHS, what types of treatments, therapies, services, products or equipment have you used to manage your child's inherited metabolic disease?	Check all that apply; specify further
For each item checked:	
OVER THE PAST 12 MONTHS, how hard was it to get [treatment, therapy, service, product, equipment]?	4-point Likert type scale
OVER THE PAST 12 MONTHS, did you get ENOUGH of [treatment, therapy, service, product, equipment]?	Got enough | Did not get enough
How difficult is it for you to manage this aspect of your child's care at home?	4-point Likert type scale
OVER THE PAST 12 MONTHS, were there services, therapies or products that your child needed to manage their IMD that you could not get WHEN they needed it?	Check all that apply
For each item checked:	
Why were the necessary medication or drugs not available when needed? Were the reasons:	Financial | Non-financial | Both
Financial or both: Please specify the FINANCIAL reasons why the [items] were not available.	Check all that apply
Non-financial or both: Please specify the NON-FINANCIAL reasons why the [items] were not available.	Check all that apply

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Over the past 3 months, how much time has your family (ALL TOGETHER) spent talking/writing to insurance companies or government agencies about health insurance coverage or reimbursement for medical products?	Select time band from list
If medical diet products used: Where do you TYPICALLY order your child's medical diet products (e.g. formula, supplements, medications, special foods) from?	Check all that apply
How do you typically RECEIVE your child's medical diet products?	Select one from list
How much time PER WEEK on average do you spend on getting medical diet products for your child (including ordering and pick up time)?	Select time band from list
Overall, how satisfied are you with the process of getting special diet products for your child?	5-point Likert type scale
Is there anything in particular that you like or dislike about your typical experiences with getting medical diet products? (Optional)	Open text
Does your family need to spend extra time planning and preparing special meals because of your child's inherited metabolic disease?	Yes / no
How much EXTRA time per WEEK on average does your family spend planning and preparing meals because of your child's inherited metabolic disease?	Select time band from list
If devices or therapies used, for each device or therapy:	
How much time PER WEEK on average does your family spend helping your child?	Select time band from list
Support services for family members	
OVER THE PAST 12 MONTHS, what type of support services for FAMILY MEMBERS have you used?	Check all that apply
For each service used:	
OVER THE PAST 12 MONTHS, how hard was it to get [service]?	4-point Likert type scale
OVER THE PAST 12 MONTHS, did you get ENOUGH of [service]?	Got enough | Did not get enough
Who helped your family to access [service] or told you about the service?	Check all that apply
OVER THE PAST 12 MONTHS, are there family support services that your family needed that you could not get WHEN you needed it?	Check all that apply
The impact of the inherited metabolic disease on caregivers' paid work outside the home	
OVER THE PAST 12 MONTHS, how many DAYS in TOTAL have your child's primary caregivers missed paid work due to caring for your participating child, for any reason?	Select one from list
Have any of your child's primary caregivers ever LEFT or QUIT a job because of your child's inherited metabolic disease?	Yes / no
Have any of your child's primary caregivers ever had to REDUCE their paid work hours because of your child's inherited metabolic disease?	Yes / no
The financial impact of the inherited metabolic disease on your family	
Over the past 12 months, what was your TOTAL household income before tax (Canadian dollars)?	Select one from list
OVER THE PAST 12 MONTHS, did you have to buy any products (including medical foods and formulas), devices, supplies, equipment or household items in order to manage your child's inherited metabolic disease?	Yes / no
If yes: How much, in Canadian dollars, did your family pay out of pocket to buy these products in the past 12 months?	Select one from list
OVER THE PAST 12 MONTHS, did you need to make any permanent or temporary modifications or renovations to your home to accommodate your child's inherited metabolic disease?	Yes / no
If yes: How much, in Canadian dollars, did your family pay out of pocket to modify or renovate your home in the past 12 months to accommodate your child's inherited metabolic disease?	Select one from list
If yes to either question re: purchase of products / home modifications: How difficult was it for your family to afford these recent costs (home modifications and/or products)?	Select one from list
Pharmacy encounters	

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In the past 6 months, how often did your family visit the pharmacy to pick up prescribed medications, foods, formulas or products for your participating child?	Select one from list
If more than once a week: In the past 6 months, how many different pharmacies did you visit?	Select one from list
If once or more:	
Where was the pharmacy (or pharmacies) located?	In Hospital / in community
ACCESS TO CARE Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
The medication or medical products typically arrived in the right formulation, supply amount, and in appropriate containers.	5-point Likert-type scale
The typical length of time between ordering the medication/medical products and picking them up was acceptable.	5-point Likert-type scale
The typical amount of time it takes to travel to the pharmacy was acceptable.	5-point Likert-type scale
Overall, how satisfied were you with your child's typical access to care at the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your child's typical access to care at the pharmacy? (Optional)	Open text
RESPECT FOR YOUR CHILD & FAMILY	
Overall, how satisfied were you with the respect that the pharmacist and/or staff typically showed you and your child over the past 6 months?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the respect that the pharmacy team typically showed you? (Optional)	Open text
COORDINATION OF CARE	
Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
Typically, the pharmacy team seemed to agree with each other about my child's treatment.	5-point Likert-type scale
Typically, the pharmacy team and providers at other locations coordinated my child's treatment appropriately.	5-point Likert-type scale
Did the metabolic clinic give you a letter about your child's inherited metabolic disease to share with the pharmacy?	Yes / no
Overall, how satisfied were you with the way that the pharmacy team typically coordinated your child's care?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the way that the care provider(s) typically coordinated your child's care? (Optional)	Open text
INFORMATION SHARING	
Overall, how satisfied were you with the typical information sharing by the pharmacy team over the past 6 months?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the typical information sharing by the pharmacy team? (Optional)	Open text
FAMILY INVOLVEMENT	
Overall, how satisfied were you with your family's typical involvement in your child's care at the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your family's typical involvement in your child's care at the pharmacy? (Optional)	Open text
FOLLOW UP AND CONTINUITY OF CARE	
Thinking about your visits and interactions with the pharmacy over the past 6 months, how much do you agree with the following statements:	
Typically, I got enough written information from the pharmacy about possible side effects of any new medications or any other new information I needed to take care of my child at home.	5-point Likert-type scale
Typically, I knew what to do or whom to call if I had any questions after leaving the pharmacy.	5-point Likert-type scale
Overall, how satisfied were you with the typical follow-up and continuity of care after visits to the pharmacy?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the typical continuity of care and follow-up after visits to the pharmacy? (Optional)	Open text

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OVERALL IMPRESSIONS OF THE PHARMACY	
Overall, how satisfied were you with your typical experiences with the pharmacy over the past 6 months?	5-point Likert-type scale
Is there anything else that you particularly liked or disliked about your typical experiences with the pharmacy? (Optional)	Open text

Indented questions are branched – only appear if specified responses to previous question(s) selected

D. Pre-questionnaire for the weekly diaries – sample questions

Question	**Response options**
Does your family do blood draws at home as part of managing your child's health?	Yes / no
If yes: How often do you and your child do blood draws at home?	Select one from list
Typically, what type of health care providers do you and your child interact with while getting the supplies, doing the blood draw, sending the sample, and waiting for and getting results?	Check all that apply
Where do you typically get the lancets you need for the blood draws?	Check all that apply
Considering your and your child's TYPICAL experience of doing blood draws at home, how much do you agree with the following statements:	
ACCESS TO CARE	
It is easy to get the items that we need to do the blood draws.	5-point Likert-type scale
If I have questions or concerns about doing a blood draw, I am able to contact the right care provider in a timely manner.	5-point Likert-type scale
The method we have to use to send the blood samples to the lab is acceptable (i.e. send by post, drop off in person).	5-point Likert-type scale
PHYSICAL COMFORT	
I receive enough support from the health provider(s) to make my child as physically comfortable as possible (i.e. to handle physical pain or discomfort) during the blood draw(s).	5-point Likert-type scale
EMOTIONAL SUPPORT	
If I share any concerns with the health care providers or staff, they respond appropriately.	5-point Likert-type scale
If my child shares any concerns with the health care providers or staff, they respond appropriately.	5-point Likert-type scale
We can do blood draws at a convenient time of the day for my family.	5-point Likert-type scale
I am comfortable drawing the blood from my child at home.	5-point Likert-type scale
INFORMATION SHARING	
I am able to share the information that I want to share about my child's blood draws with relevant provider(s).	5-point Likert-type scale
If I share information about my child's health, the care providers listen to what I have to say and respond appropriately.	5-point Likert-type scale
A care provider gives me information that I can understand about how to do the blood draw(s) at home, including getting supplies, doing the blood draw(s), and sending blood samples to the lab.	5-point Likert-type scale
A care provider gives me as much information as I want about the blood test results and clearly explains any recommendations for follow up.	5-point Likert-type scale
FOLLOW UP OF CARE	
The method that the clinic uses to send us the results of the blood tests is acceptable.	5-point Likert-type scale
The wait time for results from the blood tests is acceptable.	5-point Likert-type scale
Typically, how many days do you wait between sending the sample and receiving the results of the tests done on the blood draw?	Select one from list
YOUR FAMILY'S TIME INPUTS & FINANCIAL IMPACTS	
Typically, how much time do you and your child spend on EACH blood draw?	Select one from list

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Typically, do any of your child's caregivers have to take time off paid work to do a blood draw at home?	Yes / no
If yes: Typically, how much time off from paid work do your child's caregivers need to do a blood draw at home?	Select one from list
Does your family typically have any financial expenses that you have to pay directly because of, or related to, the blood draws you do at home, even if you are later reimbursed by an insurance plan?	Yes / no
If yes: What financial expenses does your family typically have?	Check all that apply
How much do you typically have to pay out of pocket and will NOT be reimbursed by a provincial or private insurance plan?	Select one from list
How much do you agree with this statement: The financial expenses related to doing blood draws at home typically cause me stress or anxiety.	5-point Likert-type scale

Indented questions are branched – only appear if specified responses to previous question(s) selected

E. Weekly diaries – sample questions

Question	**Response options**
Did your child receive any medical health care in Canada between [start_date] and [end_date]?	Yes / no
If yes: What types of health care encounter(s) did your child have during this week?	Check all that apply
FOLLOW-UP ON RECENT TESTS (if applicable)	
Did you expect a care provider to discuss the results of a medical test that your child had last week, in person, by phone or by e-mail?	Yes / no
If yes: With whom were you expecting to discuss the test results?	Select one from list
What type of test(s) were you waiting for the results of?	Check all that apply
How many days did you wait for a care provider to discuss the results for [test] with you?	Select one from list
How much do you agree with the following statement: The wait time for the [test] results was acceptable.	5-point Likert-type scale
How much do you agree with the following statement: A care provider gave me as much information as I wanted about the [test] results and clearly explained any recommendations for follow-up.	5-point Likert-type scale
If still waiting: How many days have you waited so far for a care provider to discuss the results for [test] with you?	Select one from list
How much do you agree with the following statement: The wait time so far for the [test] results is acceptable.	5-point Likert-type scale
How much do you agree with the following statement: A care provider gave me as much information as I wanted about where, when, and how I will get the [test] results.	5-point Likert-type scale
COVID-19	
Did your child get a COVID-19 test between [start_date] and [end_date]?	Yes/no
Was your child diagnosed with COVID-19 between [start_date] and [end_date]?	Yes/no
Were any health care encounters originally scheduled between [start_date] and [end_date] cancelled or delayed by the clinic or provider?	Yes/no

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Between [start_date] and [end_date], did you avoid seeking care for a health concern for your child due to the pandemic?	Yes/no
For every in-person encounter (questions and responses tailored to each care setting):	
Where did you and your child have this IN-PERSON care encounter?	At the Hospital / in community
Hospice or palliative care unit: Did your child stay overnight?	Yes/no
Was this encounter unplanned or pre-planned?	Select one from list
Were you and your child familiar with this place (e.g. clinic, lab, Hospital unit)?	Yes/no/somewhat
Was this place (e.g. clinic, lab, Hospital) in your province or territory of residence?	Select one from list
When did this encounter take place?	Date
During this care encounter, what type of health care provider(s) did you or your child see or communicate with?	Check all that apply
For each checked provider: Was this health care provider familiar with your child?	Yes/no/somewhat
Was this health care provider (or each of these health care providers or staff) familiar with your child's IMD?	Yes/no/somewhat
Lab: What type of tests did your child have during this encounter?	Check all that apply
If the participant identified this encounter's setting as a place where their child has frequent encounters in the Pre-Questionnaire for the weekly diaries:	
COMPARING THIS ENCOUNTER TO YOUR TYPICAL ENCOUNTERS	
Was this encounter the SAME as your typical encounters at [setting] in the following ways:	Check all that apply
The time you typically spend on encounters at [setting]: [participant response on Pre-Questionnaire]	
How long it took you to travel to the [setting]: [participant response on Pre-Questionnaire]	
Whether you or any of your child's other caregivers typically need to take time off paid work for encounters at [setting]: [participant response on Pre-Questionnaire]	
The time off paid work that you or any of your child's other caregivers typically need to take for encounters at [setting]: [participant response on Pre-Questionnaire]	
Whether your child typically misses school for encounters at [setting]: [participant response on Pre-Questionnaire]	
The time away from school that your child typically needs for encounters at [setting]: [participant response on Pre-Questionnaire]	
Your response to the statement "We are usually able to go to the [setting] at a convenient time in the day for our family": [participant response on Pre-Questionnaire]	
The time your child typically spend on encounters at [setting] (including arranging, the actual encounter, and any follow-up): [participant response on Pre-Questionnaire]	
For any aspect unchecked, the participant is asked about the aspect for this encounter.	

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Tests at the hospital laboratory (during overnight stays at the hospital, if applicable)	
During this hospital stay, did your child leave the [setting] to go to another area of the Hospital for medical testing? (e.g. radiology, imaging, diagnostics)	Yes/no
Yes: Did you or another caregiver go with your child when they had these tests?	Yes, always/yes, sometimes/no
If yes, always or sometimes: What type of tests did your child have outside the [setting]?	Check all that apply
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: We did not wait too long in the lab's waiting room.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: At the lab, information about the test process was shared with me in a way that I could understand.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: At the lab, age-appropriate information about my child's test process was shared with my child in a way that THEY could understand.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child had physical pain or discomfort during the test process, the lab's care provider(s) took the concern seriously and tried to address it.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child had physical pain or discomfort during the test process, the lab's care provider(s) respected my family's knowledge about how to make my child more comfortable.	5-point Likert-type scale
Considering ALL your child's visits to labs for medical testing during their stay at the [setting], how much do you agree with the following statement: If my child or I shared any concerns with the lab's health care providers or staff, they responded appropriately.	5-point Likert-type scale
Access to care	
How much do you agree with the following statements:	
We were able to schedule the encounter to take place at a convenient time in the day for my family.	5-point Likert-type scale
The length of time between getting a referral or scheduling the encounter and the date of the encounter was acceptable.	5-point Likert-type scale
The time it took to travel to the encounter was acceptable.	5-point Likert-type scale
We did not wait too long in the waiting room.	5-point Likert-type scale
The time spent waiting for the care provider was acceptable	5-point Likert-type scale
I was able to meet with the provider(s) I needed to talk to about my child's care.	5-point Likert-type scale
I (and/or my child) spent enough time with the health care provider(s).	5-point Likert-type scale
How long did it take you to travel from your home to this encounter?	Select one from list
Was this care encounter re-scheduled from a previous time that was cancelled or postponed?	Yes/no
Who cancelled or postponed the original encounter?	Select one from list

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Overall, how satisfied were you with your child's access to care for this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about your child's access to care during this encounter? (Optional)	Open text
Coordination of care	
Did the metabolic clinic provide your family with an emergency department letter?	Yes/no
Yes: Did you share the letter with health care providers or staff at the Emergency Department?	Yes/no
How much do you agree with the following statements:	
During this health care encounter, an Emergency Department health care provider or staff read the letter and responded appropriately.	5-point Likert-type scale
During this health care encounter, the care providers seemed to work together.	5-point Likert-type scale
During this health care encounter, the care providers seemed to agree with each other about my child's care or treatment.	5-point Likert-type scale
Health care providers that we saw during this encounter and health care providers at other locations coordinated my child's care appropriately.	5-point Likert-type scale
Overall, how satisfied were you with the way that the care provider(s) coordinated your child's care during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the way that the care provider(s) coordinated your child's care during this encounter? (Optional)	Open text
Information sharing	
How much do you agree with the following statements: During this health care encounter (including during preparing for the encounter and any follow-up)...	
...information was shared with ME in a way that I could understand.	5-point Likert-type scale
...age-appropriate information about my child's treatment was shared with MY CHILD in a way that they could understand.	5-point Likert-type scale
...I was able to share the information that I wanted to share about my child's care with the provider(s).	5-point Likert-type scale
...if I shared information about my child's health, the care providers listened to what I had to say and responded appropriately.	5-point Likert-type scale
Overall, how satisfied were you with information sharing by health care providers and/or staff during this health care encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the information sharing by care providers and/or staff during this health care encounter? (Optional)	Open text
Physical comfort	
How much do you agree with the following statements: If my child had physical pain or discomfort during the health encounter...	5-point Likert-type scale
...the care provider(s) took the concern seriously and tried to address it.	5-point Likert-type scale

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...the care provider(s) respected my family's knowledge about how to make my child more comfortable.	5-point Likert-type scale
Overall, how satisfied were you with the care provider(s)'s efforts to make your child physically comfortable during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the care provider(s)'s efforts to make your child PHYSICALLY comfortable during this encounter? (Optional)	Open text
Emotional support	
How much do you agree with the following statements:	
If I shared any concerns with the health care providers or staff, they responded appropriately.	5-point Likert-type scale
If MY CHILD shared any concerns with the health care providers or staff, they responded appropriately.	5-point Likert-type scale
Overall, how satisfied were you with the health care providers' EMOTIONAL SUPPORT given to you and your child during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about efforts made by the health care providers or staff to provide EMOTIONAL support to you and your child during this encounter? (Optional)	5-point Likert-type scale
Family involvement	
Overall, how satisfied were you with your family's involvement in your child's care during this care encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the care provider(s)'s efforts to involve your family during this encounter? (Optional)	Open text
Respect for your child & family	
Overall, how satisfied were you with the respect that care providers and staff showed you and your child during this encounter?	5-point Likert-type scale
Was there anything that you particularly liked or disliked about the respect that care providers and staff showed you and your child during this encounter? (Optional)	Open text
Follow up and continuity of care	
How much do you agree with the following statements:	
Before the end of this health care encounter, I got enough written information about possible side effects of any new medications, physical limitations, dietary needs or any other new information I needed to take care of my child at home.	5-point Likert-type scale
Before the end of the encounter, a care provider explained in a way that was easy to understand what symptoms or health problems to look out for after the encounter.	5-point Likert-type scale
I knew what to do or whom to call if I had any questions after this health care encounter.	5-point Likert-type scale
I got enough information about the next steps that I needed to take after the encounter. (e.g. booking new appointments, location of follow-up appointments, renewing prescriptions)	5-point Likert-type scale
The care provider(s) took all the steps that I expected them to take after the encounter. (e.g. making referrals, booking new appointments)	5-point Likert-type scale
Overall, how satisfied were you with the continuity of care and follow-up to this encounter?	5-point Likert-type scale

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Was there anything that you particularly liked or disliked about the continuity of care and follow-up to this encounter? (Optional)	Open text
Time inputs and financial impacts	
How much time did you and your child spend on this encounter (including arranging, travel if applicable, waiting, and the actual encounter)?	Select one from list
Did your family have any financial expenses that you had to pay directly because of, or in relation to, this care encounter, even if you were later reimbursed by an insurance plan?	Yes/no
Yes: What financial expenses did your family have?	Check all that apply
How much did you have to pay out of pocket and will NOT be reimbursed by a provincial or private insurance plan? Give your best estimate.	Select one from list
How much do you agree with this statement: The financial expenses related to this health care encounter cause me stress or anxiety.	5-point Likert-type scale
Did you or any of your child's other caregivers have to take time off paid work for this care encounter?	Yes/no
Yes: ALL TOGETHER, how much time off paid work did you need for this care encounter?	Select one from list
Did your child miss school/class for this care encounter?	Yes/no
Yes: How much time away from school/class did your child need for this care encounter?	Select one from list
Overall experience	
Overall, how satisfied were you with your and your child's experiences with care during this encounter?	5-point Likert-type scale
Was there anything else that you particularly liked or disliked about your and your child's experiences with care during this encounter? (Optional)	Open text
Compared to similar encounters that took place before the pandemic (i.e., March 2020), was this encounter shorter or longer?	Select one from list
Compared to similar encounters that took place before the pandemic (i.e., March 2020), was the amount of time from when you scheduled the appointment to the date of the appointment shorter or longer?	Select one from list
Did the provider request or tell you that there was a limit to the number of caregivers who could attend the encounter with your child?	Yes/no
Yes: Did this affect who or how many people went to the encounter with your child?	Yes/no
In your opinion, was there any other important difference between this encounter and other ones like it before the pandemic? If yes, please describe below.	Open text
Was this encounter scheduled BECAUSE it was required for a study or trial that your child is taking part in?	Yes/no
Which of your child's caregivers went to this encounter with your child?	Check all that apply
Who contributed to filling out this Experience Questionnaire?	Check all that apply
Which of these people was the MAIN person filling out this questionnaire?	Select one from list
Questions similar to these are also tailored to remote/virtual encounters, and for any blood draws done at home by the family.	

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F. Care Map Interview Guide

Overall Network of Care

1. Can you please walk me through your child's network of care?
    - *Probe for specific aspects related to:*
        - Validation of listed providers and connections – is the network accurate as it is or would you like to make any changes to it?
        - The process of drawing the network of care- how did you decide who to include in the network?

Identification of Key Providers

2. You identified [provider X] as a key provider. What are the factors that make them a 'key provider' for (kid's name)?
    - *Probe for specific aspects related to:*
        - From the care map questionnaire, I noticed that you indicated that this provider knows your child very well. What does that look like to you? (how do you know?)
        - How often does (kid's name) interact with the provider?
        - What is the provider's role in the child's care?

Care Coordination

3. You identified that [provider X] and [Provider Y] are connected. Can you tell me about that connection?
    - *Probe for specific aspects related to:*
        - What is the nature of the connection?
        - What is the impact of the connection on the family? How can you tell?
4. On the care map questionnaire, you told us that [provider X] coordinates with other providers "very well."
    - How does provider x work with other providers (e.g., shares information, makes referrals, you don't have to fill them in on Can you tell me about factors that influenced your positive rating?
5. On the care map questionnaire, you told us that [provider X] coordinates with other providers "not well at all."
    - Can you tell me about factors that influenced your negative rating?
    - What could/should be done to improve it?

Adequacy of Network of Care

6. How well does this network of care meet your child's needs? How does this network of care meet your needs?
    - *Probe for specific aspects related to*
        - Are there parts of the network that work better than others? What parts work better? In what ways?
        - What can be improved in this network of care? How could the network be improved to better meet (kid's) needs?
        - Are there people who should be key providers but they are not listed as such? Who and How come?
        - Are there providers who should be connected on your care map but who are not currently connected? Which providers do you think should be connected? How would this help?

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G. Encounter Interview Guide

Direct contributors to satisfaction rating

1. You rated your satisfaction with [this encounter / specific Picker Principle] [RATING]. In your view, what made this interaction [positive/negative]?
    - *Probe for specific aspects related to:*
        - Picker Principles
        - Setting
        - Modality

Identification of how negative encounter could have been different

2. *If negative:* In your opinion, what would have made this encounter better for you and your child?
    - *Probe for role of:*
        - Specific providers / teams
        - Specific actions (actor not necessarily important)

3. *For each agent of change:* In your opinion, what could they have done differently?

Identification of HCP who could have helped

4. *If negative:* Is there another health care provider involved in your child's care who you think could have helped in this situation?

Comparison to previous, similar encounters (same mode)

5. Have you been to [setting] before? / Have you met this [provider] before?
    - *If yes:* How did this interaction compare with other interactions you've had [with PROVIDER/at LOCATION]?
        - *If worse or better:*
            - How was it worse/better?
            - Was there anything else different about this encounter than other ones (e.g., longer wait time, different receptionist)?
        - *If the same – negative:* What do you wish would happen instead?

Impact of the encounter

6. How did this interaction affect your child, you, and other members of your family?
    - *Probe for different impacts*, e.g., psychological, physical, emotional, social, financial
    - *If negative:* What / is there anything else that would have made this interaction more positive for you?
    - *If negative and other encounters are the same:* Since you've had other negative experiences [at clinic / with provider], did it change the way you prepared for this encounter?

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Supplementary material 4. Research ethics committee approvals

The study protocol and procedures were approved by:

1. Children's Hospital of Eastern Ontario Research Ethics Board (ID #1955), covering the following sites:
    a. Children's Hospital of Eastern Ontario, Ottawa, ON, Canada
    b. The Hospital for Sick Children, Toronto, ON, Canada
    c. London Health Sciences Centre, London, ON, Canada
    d. Kingston Health Sciences Centre, Kingston, ON, Canada
    e. Hamilton Health Sciences, Hamilton, ON, Canada
2. The University of Ottawa Research Ethics Board (File no. H-04-20-5757)
3. University of Calgary Conjoint Health Research Ethics Board (ID no. REB20-2225)
4. University of British Columbia C&W Research Ethics Board (No. H20-00673)
5. University of Alberta Research Ethics Board (ID no. MS3_Pro00098519)
6. Izaak Walton Killam Research Ethics Board (Project # 1025806)
7. McGill University Health Centre Research Ethics Board (No.13-331-PED (CIMDRN) / 2021-7171)
8. University of Manitoba Health Research Ethics Board (Ethics # HS24028 (H2020:291))

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